Towards developing an outpatient assessment tool of LSL that incorporates clinical evaluation and the impact on patient and family in terms of Health Related Quality of Life.

By

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Thesis submitted for the degree of Doctor of Philosophy

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I, Lindy May, confirm that the work presented in this thesis is my own. Where information has been derived from other sources, I confirm that this has been indicated in the thesis.

Dedication page

I would like to thank the children and families who participated in the study for their honesty, bravery and humour. And my husband and children, who encouraged me and believed in me during this challenging time!

There are many professionals who have helped me, in particular my supervisors Aabir Chakraborty and Jo Wray, but many more people including Suellen Walker, Adam Kuczynski, Dominic Thompson and Grazia Manzotti.

This research was undertaken at the neurosurgical unit, Great Ormond Street Hospital for Children, NHS Foundation Trust, London.

This study is in memory of my Mother who died before I could finish it. She would have been thrilled to have another Dr May in the family.

Abstract

Introduction

There is wide variation in how lumbosacral lipoma (LSL) is assessed in the clinical setting; furthermore, there is little data regarding the impact that LSL has on the Health Related Quality of Life (HRQL) of children.

Secondly, there is limited information regarding the relationship between LSL type, and clinical and HRQL outcomes.

This thesis aims to:

1. Develop an assessment tool for use in the out-patient setting, which would provide a standardised method by which to objectively assess outcomes of interventions including surgery, and allow comparison and audit across different neurosurgical units.

2. Identify the key HRQL factors that affect the child and parent.

3. Identify if there is a relationship between LSL type, and clinical and HRQL outcomes.

Method

1. A systematic review was undertaken to identify the assessment criteria in children with LSL.

2. A cross sectional analysis of a cohort of 54 children with LSL aged between 5 and 18 years of age was performed.

3. The results from points 1 and 2 were combined to develop an objective assessment tool.

4. HRQL was assessed using a number of generic questionnaires and by asking the children and parents directly, what was important to them in terms of

their disease. The results were combined to ensure all aspects of the impact of LSL were identified, including the effect of the disease on the well-being of parents.

5. The relationship between LSL type, and clinical and HRQL outcomes was systematically analysed in a cohort of 54 children, to examine the association between these symptoms and the type of LSL.

Results

1. It was evident from the systematic review that there was no consistency in the literature regarding assessment methods for this group of children. Based on this review and the prospective study, an objective tool was developed that can be easily useable in the clinical setting.

2. The clinical issues most significantly associated with HRQL were mobility, pain and urology. Urology issues were not identified by self-report from the questionnaire results, but from verbal self-reporting.

3. The transitional group have a higher risk of clinical abnormalities and deterioration and the dorsal group the least, in the majority of clinical outcomes. There was a trend for children with transitional lipomas to have a lower HRQL and the dorsal group to have the highest HRQL, on the majority of measures.

Discussion

In addition to a standardised clinical assessment tool, an appropriate HRQL questionnaire and a standardised pain assessment tool will be utilised to provide a holistic assessment of children with LSL.

The study results suggest children could be risk stratified according to LSL type, with more intensive initial investigations and potential interventions in children with transitional lipomas.

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List of abbreviations

B	
BE	Behaviour
BEH	Behavioural Adjustment
BP	Bodily Pain
BPD	Bodily Pain / Discomfort
BPNG	British Paediatric Neurosurgical Group
C	
CHQ-PF50	Child Health Questionnaire-Parent Form
CHQ-CF87	Child Health Questionnaire – Child form
CIC	Clean Intermittent Catheterisation
CiH	Changes in Health
CINAHL	Cumulative Index for Nursing and Allied Health Literature
CQUINS	Commissioning for Quality and Innovation scheme
CRD	The Centre for Reviews and Dissemination
CRN	Clinical Delivery Networks
CSF	Cerebrospinal fluid
CRG	Clinical Reference Groups
E	
EMG	Electromyography
ERIC	The Children's Bowel and Bladder Charity
F	
FA	Family Activities
FC	Family Cohesion
FIC QOL	Fecal Incontinence and Constipation Quality of Life Scale

FRE	Freedom from Anxiety
G	
G-CAT	The GOSH Clinician's Assessment Tool
GH	Global Health
GHP	General Health Perception
H	
HAD	Hospital Anxiety and Depression scale
HAP	Happiness and Satisfaction
HRQL	Health Related Quality of Life
HRQOL-SB	The Health Related Quality of Life-SpinaBifida questionnaire
I	
IASP	International Association for the Study of Pain
ICF	The International Classification of Functioning, Disability and Health
ICF-CY	The International Classification of Functioning, Disability and Health Child and Health Version
INT	Intellectual and Social Status
ISPN	International Society of Pediatric Neurosurgery
IQR	Interquartile Range
L	
LMMC	Lipomyelomeningocele
LSL	Lumbosacral lipoma
L1-L5	Lumbar spine 1 to 5
M	
MH	Mental Health
MRC	Medical Research Council

MRI	Magnetic Resonance Imaging
N	
NEM	Necker-Enfants- Malades
NHS	National Health Service
NICE	National Institute for Health and Care Excellence
NNAP	Neurosurgical National Audit Programme
NRES	National Research Ethics Service
O	
ODN	Operational Delivery Networks
P	
PAQ-A	Physical Activity questionnaire – Adolescent version
PAQ-C	Physical Activity questionnaire – Child version
PCF	Paediatric Continence Forum
PE	Parent Emotion
PedsQL	The Pediatric Quality Of Life Inventory
PF	Physical Functioning
PH2	Piers- Harris Children's Self Concept Scale
PsS	Physical summary Score
PHY	Physical Appearance and Attributes
PICO	Population, Intervention, Comparison, Outcomes
PIP	The Pediatric Inventory for Parents
POP	Popularity
pnbg	National Paediatric Neuroscience Nursing Benchmarking Group
PedsQL PPQ	The Pediatric Quality Of Life Inventory Pain Questionnaire

PROMS	Patient Related Outcome Measures
PsS	Psychological summary Score
PsycINFO	American Psychology Association publications and database
PT	Parent Time
Q	
QOL	Quality of Life
R	
Rb	Role limitations- behavioural
R&D	Research and Development
REC	Research and Ethics Committee
Re	Role limitations- emotional
Rp	Role limitations-physical
S	
SBNS	Society of British Neurological Surgeons
SE	Self Esteem
SHINE	SpinaBifida Hydrocephalus Information Networking Equality association
S1 to S5	Sacral spine I1 to 5
SPSS	Statistics Package for the Social Sciences
W	
WISC_R	The Wide Range Achievement Test-Revised

Preface

Background to the study

Lumbosacral lipoma (LSL) is a rare and complex congenital condition and the long-term outcome for the child is variable, and dependent on the underlying pathoembryology and physiology, inconsistent natural history of the disease, the criteria used to intervene surgically, and the surgical strategies employed. Not all children develop symptoms, however in those that do, changes may be irreversible, especially urological deterioration (Pierre-Kahn et al., 1997, Wykes et al., 2012). Optimal management therefore would include standardised assessment tools by which the clinician could risk stratify patients and intervene as appropriate. Furthermore, the assessment tool would be used for ongoing monitoring of patients who have undergone surgery.

The assessment tool would also provide the clinician with the opportunity to not only follow the outcomes of individual patients over many years, but also compare different series of patients, who may be undergoing different management strategies or are being managed in different neurosurgical units. NHS England highlights the importance of transparency in sharing and standardising health outcomes. The NHS Outcomes Framework (Department of Health, 2014 / 2015) which arose from the Darzi report (Department of Health, 2008) highlights the importance of also evaluating and managing the Health Related Quality of Life (HRQL) of individuals with chronic conditions and the effect this has on their carers.

HRQL describes an individual's perceived ability to participate in physical and social activities in their environment and their level of enjoyment or satisfaction in that involvement given their disease or health status (Eiser & Morse, 2001). HRQL measures help determine the impact of chronic disease in terms of the efficacy of interventions, evaluation of outcomes and to help identify the requirement for service provision (Eiser and Morse, 2001).

LSL is a chronic disease with associated morbidity, and management is aimed at maximising health and HRQL, and minimising disability over the spectrum of the child's life. There are currently no standardised assessment tools by which to assess the clinical and HRQL outcomes of children with LSL.

Aims

The two general aims of this thesis were to:

1. Develop a tool for assessment of children with lumbosacral lipoma (LSL) in the outpatient setting. It is hypothesised that this assessment tool will:

    a. Provide a standardised method of continual surveillance of the child including Health Related Quality of Life (HRQL).

    b. Provide a standardised method by which to objectively assess outcomes of intervention including surgery.

2. Describe a preliminary analysis for determining if there is a relationship between lipoma type and clinical and HRQL outcomes. In addition to providing a more thorough understanding of this rare condition, this may assist in the evaluation of prognosis and inform a more selective treatment policy. Data was also collected to identify what particular aspects of quality of life appeared to be important to the child.

In addition, this thesis describes a preliminary analysis of the impact of LSL on the parents of children with LSL.

Steps taken to achieve these aims included:

1. Undertaking a systematic review (SR) to identify the salient clinical signs and symptoms on which to base decision making regarding interventions including surgery.

2. Objectively assessing a group of children with LSL using standardised methods of clinical assessment.

3. Evaluating the child's Health Related Quality of Life (HRQL) and the effect that living with this chronic disease has on the child. This was divided into the use of formalised questionnaires and by asking the children what was important to them in terms of their disease.

4. Distilling the study findings from aims 2) and 3) into a tool which can be used within the outpatient setting to provide a consistent and overall assessment to provide a basis for future management.

5. Distilling the study findings from the HRQL questionnaires and the replies from the children to provide a more holistic view of the burden of the disease. This would also provide a basis for a management plan.

6. Identifying if there is a relationship between lipoma type, and clinical and HRQL outcomes.

Formalised questionnaires were used to explore the impact of LSL on the parents of children with LSL; in addition, the parents were asked what was important to them in terms of their child's disease.

Thesis Outline

The thesis is comprised of 8 chapters. Chapter 1 forms the introduction to the thesis. A description of the pathoembryology of LSL within the spectrum of spinal dysraphism is presented, followed by the pathophysiology of clinical deterioration in children with LSL and its clinical correlate. The importance of standardising methods of assessment both in terms of clinical practice and how the child's HRQL may be affected is highlighted.

Chapter 2 provides a systematic review of the current publications of LSL in children and the tools by which children are assessed, both with regard to their clinical symptoms and their HRQL.

Chapter 3 describes the methodology for the clinical study of 54 children with LSL including the ethical requirements of the study, participant selection, methods of data collection and data analysis, looking specifically at relationships between clinical assessment, underlying pathophysiology and HRQL. In addition, clinical outcomes in children with specific types of LSL are assessed to determine if longer-term prognosis could be suggested for specific LSL types.

Chapters 4 and 5 provide an analysis and findings of the clinical variables and HRQL respectively of children with LSL, with the aim of distilling the results of this study into an assessment tool, which can be used in the clinical setting.

Chapter 6 provides an insight into the issues that are important to the child with LSL and their parents. An assessment of the effect that living with a child with LSL has on the parents, is discussed in Chapter 7. The final chapter will discuss why this approach should be validated and used as the basis for comparing outcomes of individual patients and outcomes between units. This will include a wider discussion of the requirement from NHS England for standardising care across paediatric neurosurgical settings. Finally, whilst acknowledging the limitations of the study, the implications for clinical practice and future research are discussed.

Chapter 1. Lumbosacral Lipoma (LSL).

1.1. Introduction

Spinal dysraphism is a general term for a family of congenital malformations of the spine and spinal cord and covers a wide spectrum of spinal abnormalities. Spinal dysraphism is described in literature of the Middle Ages, although the mythological figure of the Satyr, with associated equinovarus feet and lumbosacral hypertrichosis was recognised even earlier (Daszkiewicz et al., 2007).

Figure 1.1 Satyr Portrait

https://www:zealousgears.com (Google images)

1.2. Nomenclature

Closed spinal dysraphism (spinabifida occulta) encompasses a heterogeneous group of disorders altering the normal development of the terminal spinal cord.

1.3. Clinical implications

Lumbosacral lipomas (LSL) represent the most common type of occult spinal dysraphism and occur with a frequency of 1:4,000 live births, of which 54-86% are found in the conus medullaris (Pierre-Kahn et al., 1997).

1.4. Epidemiology

Lumbosacral lipoma (LSL) is a complex and diverse condition and the natural history of the disease remains unknown, making comparison between patients, between management strategies, and offering a prognosis, a difficult process.

1.5. Clinical outcomes

LSL usually presents as a lumbosacral mass, obvious at birth. The long- term outcome for the child is variable and dependent on the underlying pathophysiology and natural history of the disease. However, the degree of clinical dysfunction is variable. Deterioration may be due to tethering of the spinal cord or intrinsic dysplasia and encompasses urological, neurological and orthopaedic deficits (Thompson, 2010a). The diagnosis of tethered cord syndrome remains primarily a clinical one, requiring evaluation throughout childhood and beyond (May et al., 2013).

Muthukumar (2009) attempts to refine the poor nomenclature of LSL and suggests that congenital spinal lipomatous malformations can be separated into 2 groups: Group I consisting of lipomas without dural defect, including filum lipomas, caudal lipomas without dural defect, and intramedullary lipomas; group 2 consisting of lipomas with dural defect including dorsal lipomas, caudal lipomas with dural defect, transitional lipomas, lipomyeloceles, and lipomyelomeningoceles (Muthukumar, 2009a). Chapman (Chapman et al., 1999) and Pang (Pang, 1986) also provide details of the pathoembryology and physiology of the spinal cord in relation to spinal lipomas and this is provided in more detail in the following sections.

The clinical outcomes for children with LSL are highly variable with as many as 30% of children in group 2 presenting as asymptomatic (Thompson, 2010a). However as more than 50% of children in group 2 will deteriorate over time and this deterioration may be irreversible, the thesis will focus on children with group 2 lipomas with the aim of providing a standardised assessment tool by which to monitor these children.

1.6. Pathoembryology and physiology of the spinal cord.

Spinal dysraphism describes a spectrum of spinal cord anomalies. Heterogeneous embryological mechanisms have been proposed with regard to the spectrum but these are largely unproven. The following description of embryology provides a framework on which the anomalies, including LSL may be more fully understood.

The process of embryogenesis has three stages as described below:

1.6.1. Gastrulation.

The biologist Lewis Wolpert, a specialist in embryology, provided the following frequently cited quote in 1986:

“It is not birth, marriage, or death, but gastrulation, which is truly the most important time in your life." (Wolpert, 1986).

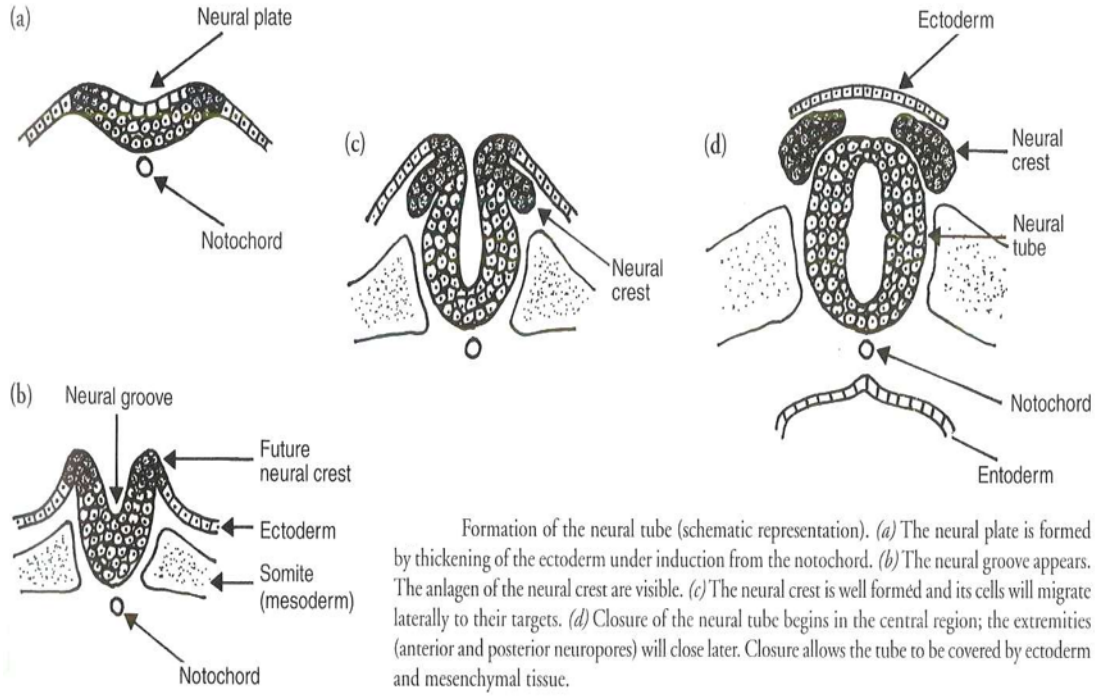
Gastrulation begins with the movement of primitive cells known as epiblasts, towards the dorsal midline of the embryo to become the primitive streak, which eventually extends from the cranial to the dorsal part of the embryo. This migration of cells results in the genesis of three germ layers comprising the following:

- Ectoderm: This forms the Central Nervous System and the skin
- Mesoderm: This forms the musculoskeletal system
- Endoderm: This forms the alimentary canal

The primitive streak regresses caudally as the embryo enlarges with a resulting primitive midline tube known as the notochord.

1.6.2. Neurulation

The process of neurulation results in the formation of the spinal cord and brain. Neurulation begins with the thickening of the ectoderm and the resulting formation of the neural plate.



Formation of the neural tube (schematic representation). *(a)* The neural plate is formed by thickening of the ectoderm under induction from the notochord. *(b)* The neural groove appears. The anlagen of the neural crest are visible. *(c)* The neural crest is well formed and its cells will migrate laterally to their targets. *(d)* Closure of the neural tube begins in the central region; the extremities (anterior and posterior neuropores) will close later. Closure allows the tube to be covered by ectoderm and mesenchymal tissue.

Figure 1.2 Formation of the neural tube.
(Aicardi et al., 2009).

There are two stages of neurulation:

- *Primary neurulation*: 0 to 27 days gestation.

The ectoderm layer of the embryo is influenced by the notochord with two longitudinal folds evolving, which fold inwards until the edges come in contact and fuse. This closure of the neural tube consists of several “waves”, commencing at the cervicomedullary junction, the rostral end forming the brain and the caudal end becoming the spinal cord and canal.

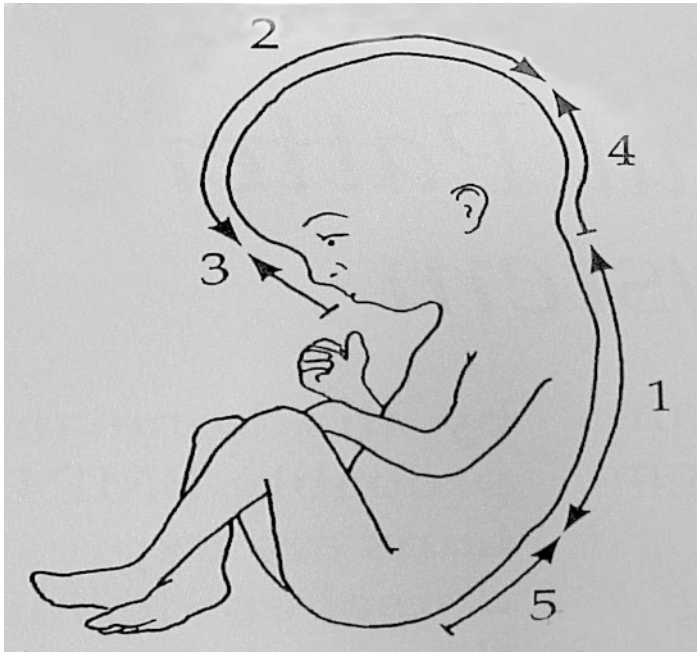


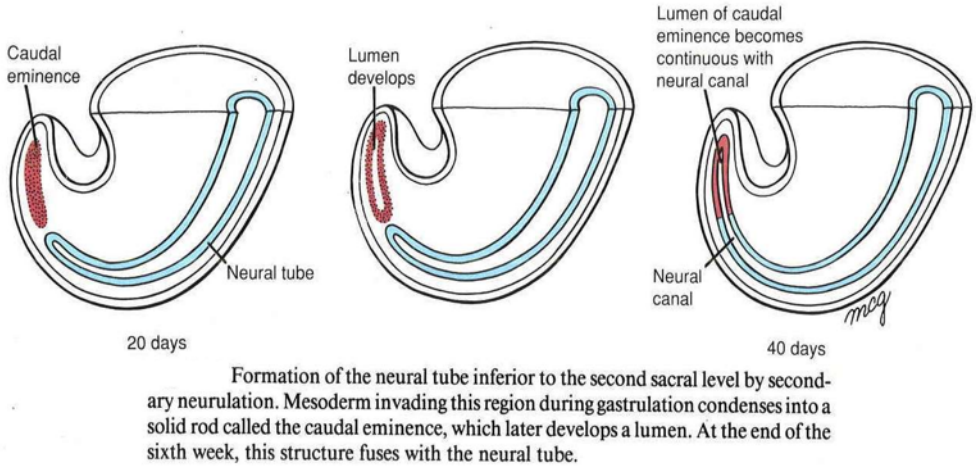
Figure 1.3 Phases of neural tube development
(Hamilton et al., 1976)

Abnormalities in primary neurulation result in myelcoele and myelomeningocele, whereby the spinal vertebrae are not fully formed and remain open, thus allowing a proportion of the spinal cord to protrude through (Spinabifida Aperta).

- *Secondary neurulation (retrogressive neurulation; from day 27 – 42 of gestation)*

The mass of cells in the tail bud of the embryo known as the caudal cell mass, will form the caudal end of the spinal cord (distal to sacral vertebra 2: S2) and will also contribute to the genitourinary tracts and gastrointestinal system. The

cells in the caudal cell mass join the cells formed by primary neurulation to form a complete spinal cord in a process known as secondary neurulation. The terminal part of the spinal cord involutes with growth and regression of the tail bud, leaving the conus and filum terminale.



Formation of the neural tube inferior to the second sacral level by secondary neurulation. Mesoderm invading this region during gastrulation condenses into a solid rod called the caudal eminence, which later develops a lumen. At the end of the sixth week, this structure fuses with the neural tube.

Figure 1.4 Red: site of secondary neurulation
(Newman et al., 2005)

In summary, all neural tissue at and cranial to the level of S2, is likely to be derived from primary neurulation. Tissues caudal to S2 are likely to be derived from secondary neurulation.

1.6.3. Dysjunction

When neural tube closure is complete the ectoderm destined to be skin separates from that destined to be neural, in a process known as dysjunction.

McLone suggests that spinal lipomas might result from an error in dysjunction, premature disjunction occurring when the developing neural tube separates from the cutaneous ectoderm prior to complete neural tube closure, with mesenchymal cells entering the spinal cord where they differentiate into fat

(McLone, 2001). However, this process does not explain caudal and transitional lipomas of the conus as this process occurs during secondary neurulation.

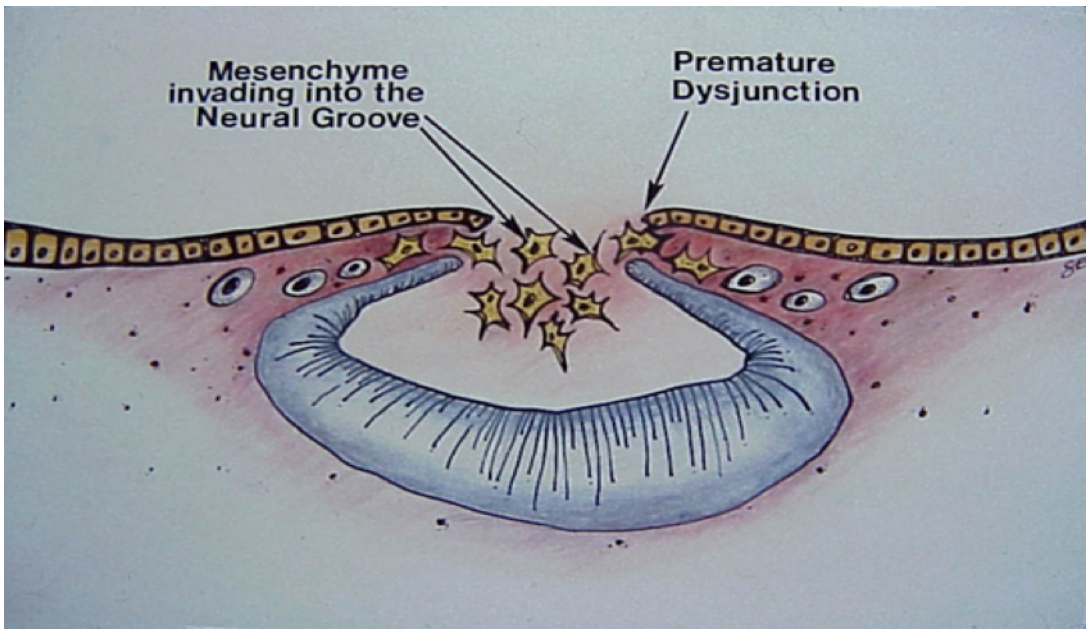


Figure 1.5 Dysjunction
(Pang, 1986)

The resulting skin covered fatty mass includes intradurallipoma, lipomyelocele and lipomyelomeningocele.

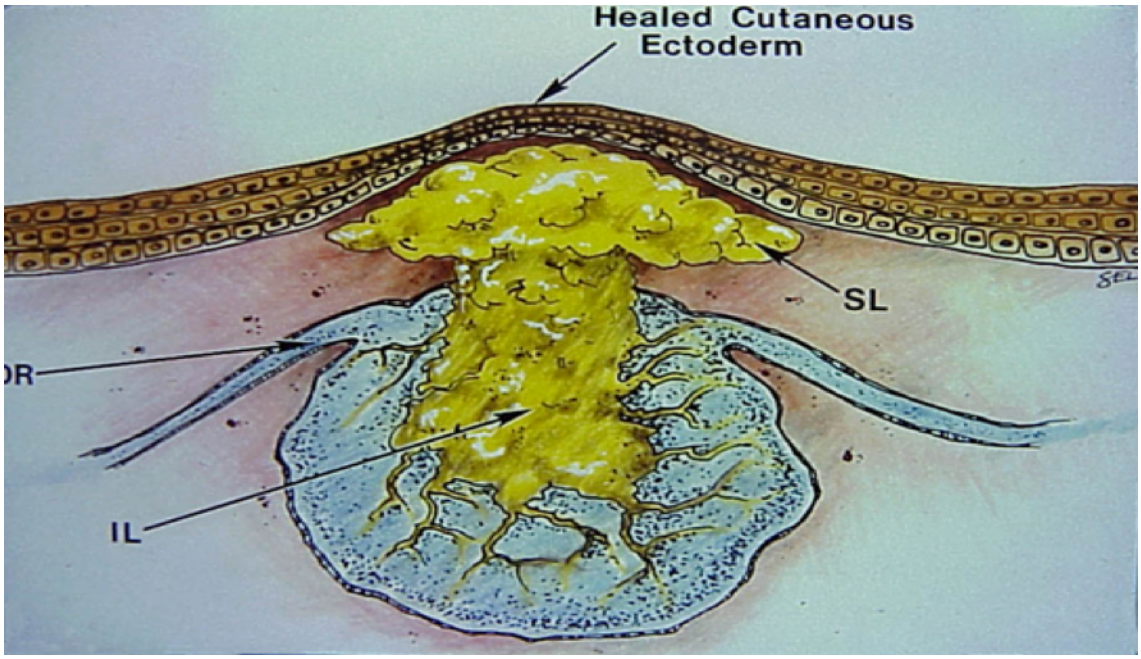


Figure 1.6 Incomplete Dysjunction

IL: intramedullary lipoma; SL: subcutaneous lipoma; R: dorsal roots. (Pang, 1986)

Incomplete dysjunction results in a persistent connection between the neural and superficial ectoderm and results in a different group of dysraphic anomalies that includes dermal sinus tracts and meningoceles.

1.6.4. Spinal cord growth

The spinal cord grows at a slower rate than the vertebral column and this results in the progressive ascent of the conus medullaris. By 40 weeks of gestation the conus is at the level lumbar vertebra 3 and by the age of 2 months has reached the adult level of lumbar vertebra 1 to 2. Some authors suggest the conus is already at the “adult” position at the time of birth: Tubbs and Oakes (Tubbs and Oakes, 2004) suggest that there is no “normal” position of the conus but merely a normal range, and that clinicians should base their practice on clinical rather than purely radiological findings.

The normal spinal cord is suspended in cerebrospinal fluid and attached to the surrounding spinal canal by the delicate dentate ligaments and by the nerve roots. There is thus provision for spinal cord movement with changing spinal growth or movement. However, if the cord is tethered this movement is not possible and traction of the conus and resulting ischaemia can occur (Yamada et al., 2007).

In addition to tethering associated with a spinal lipoma, tethering can occur in association with a thickened filum terminale. This abnormality does not form part of the study research, but is provided for completion of the description of spinal dysraphism and tethered cord.

1.6.5. Thickened / Fatty filum terminale

In many cases the filum is surrounded by normal nerve roots and the clinical outcomes are correspondingly better for children with filum terminale, than children with spinal lipoma (Pierre-Kahn et al., 1997). Blount and Elton (Blount and Elton, 2001) suggest that the lack of classification for abnormalities of the filum leads to an incomplete understanding of the natural history of these lesions and that the relevance of the thickness of the filum for example, is unclear. They suggest these lesions could be classified as follows:

7. Fatty filum with descended position of the conus and symptomatic child (signs of tethered cord).

8. Fatty filum with descended position of the conus and asymptomatic child.

9. Fatty filum with normal position of the conus and symptomatic child (signs of tethered cord).

10. Fatty filum with normal position of the conus and asymptomatic child.

The authors suggest that such a classification of these lesions will facilitate correct treatment and outcomes, recognising the potential risks of surgical intervention to an asymptomatic child. Although the risk for surgical intervention

of filum lipomas is lower than for that lipomas of the conus, careful consideration is required to ensure that the risks of surgery outweigh the risks of managing the child conservatively (Blount and Elton, 2001).

1.7. Cutaneous manifestations and lipomatous fat

An angioma, hairy patch, dermal sinus, dimples and appendages can be present in children with LSL (Hoffman, 1987, Kanev and Bierbrauer, 1995, Pierre-Kahn et al., 1997) and a subcutaneous mass of fat is present in over 90% of patients (Blount and Elton, 2001). The subcutaneous mass is often asymmetrical, with neurological deficits often greatest on the ipsilateral side to the mass (Blount and Elton, 2001). McLone (McLone D, 2001) suggests that lipomas increase in size in line with other body fat deposits for example during adolescence. Tethered cord is often associated with a period of spinal growth with age and it is possible increased fat deposition may add to the tethering process. It is suggested that adipocytes increase in size with age, with the proportion of fat increasing from 14% to 25% at 6 months (Rolland-Cachera M et al., 1971). A more recent study suggests that adipocyte size increases significantly in the first year of life, with a significant increase during adolescents, particularly amongst females (KOHL, 2013.)In a publication by Pierre- Kahn and colleagues, an increase in lipoma size was identified in 13.2% of the study group of patients with spinal lipoma, but the authors acknowledged there was an associated increase in the body fat pool due to obesity or pregnancy, and suggested further research was warranted to identify if there is an association between growth and lipomatous mass (Pierre-Kahn et al., 1997).

Figure 1.7 Lumbosacral lipomatous mass
(Guggisberg et al., 2004)

Very occasionally abnormalities of the perineal region are seen including abnormal genitalia, imperforate anus and atrophy, reflecting common origin in the caudal cell mass (Kanev and Bierbrauer, 1995, Mielke et al., 1982, Pierre-Kahn et al., 1997).

1.8. Classification of lumbosacral lipoma (LSL)

Chapman (Chapman, 1982) describes an anatomically based classification of spinal lipoma, whereby the position of the neural placode (the point of attachment of the lipoma to the spinal cord) is described in relation to the conus and the posterior nerve roots. The classifications he describes are presented below:

1.8.1. Caudal lipoma

- The tip of the conus is continuous with the lipomatous mass, which extends to the terminal thecal sac.
- Some of the lower sacral roots may be involved.

(Chapman, 1982)

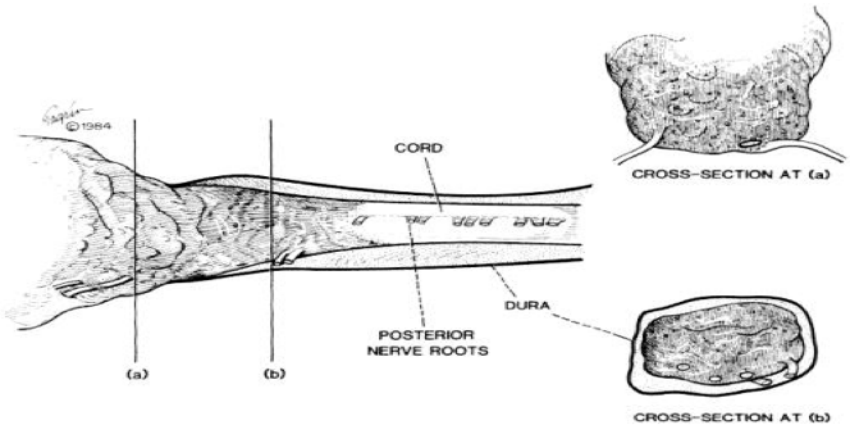


Figure 1.8 Diagrammatic representation of a caudal lipoma.
(Chapman et al., 1999)

1.8.2. Dorsal lipoma

- Nerve roots emerge anterior to the zone where the lipoma, dura, and conus meet.
- Associated fatty mass penetrates a fascial defect.
- Extends into the spinal cord and may be asymmetrical, resulting in cord rotation.

(Chapman, 1982).

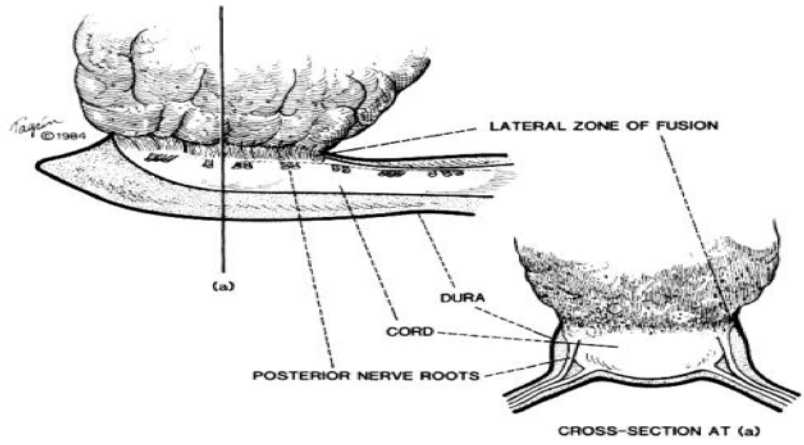


Figure 1.9 Diagrammatic representation of a dorsal lipoma.
(Chapman et al., 1999)

1.8.3. Transitional lipoma

- Comprises components of both dorsal and caudal variants of lipomas (thus the dorsal surface and tip of the conus and the cauda equina are involved).
- Rotation of the cord produces forward displacement and shortening of the nerve roots (Wykes et al., 2012) with more deficits, in particular neurological deficits, on the side of the lipoma (Lazareff, 2011).

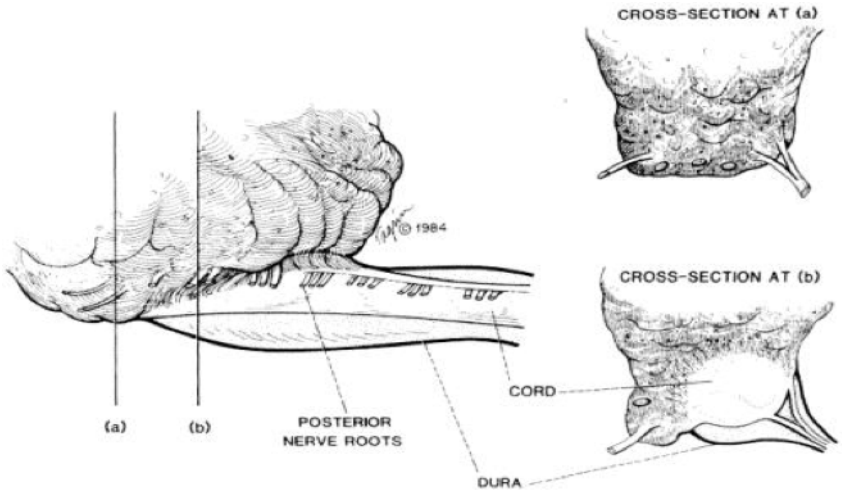


Figure 1.10 Diagrammatic representation of a transitional lipoma.
(Chapman et al., 1999)

1.8.4. Chaotic lipoma

- Confusing blend of the ventral fat and neural placode.
- Engulfs neural tissue and nerve root.
- May be associated with sacral agenesis.

(Pang et al., 2009)

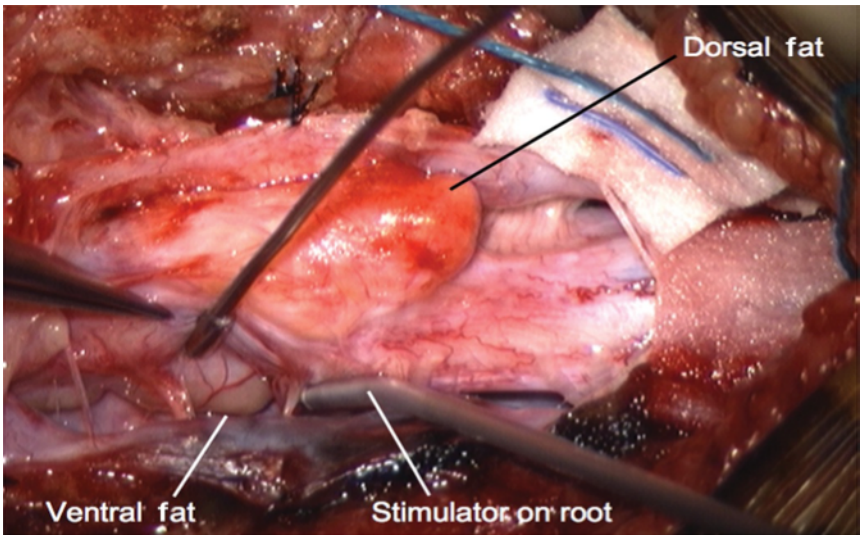


Figure 1.11 Photo depicting chaotic lipoma

Ventral pia covered fat medial to ventral nerve root, and dorsal fat perched on dorsal side of placode. (Pang et al., 2009)

1.9. The Pathophysiology of deterioration

Clinical function may be compromised due to three possible causes:

1. Intrinsic dysplasia of the spinal cord and nerve roots.
2. Mass effect from the LSL causing compression of the spinal cord and nerve roots.
3. Spinal cord tethering with resulting stretching, ischaemia and damage to the spinal cord and nerve roots. Yamada and colleagues used animal models to study the tethering process and demonstrated reduced mitochondrial oxidative metabolism and ischaemia associated with tethered cord, which could be corrected with surgical untethering (Yamada et al., 1995).

1.9.1. Tethered cord

Some children have static neurological deficits that are apparent from birth and only become more obvious as the child grows. The aim of surgical intervention

is to stabilise, not reverse the neurological deficit. However, deterioration of neurological function with age is thought to be secondary to increased stretch on the spinal cord, with axial growth spurts resulting in tethering of the spinal cord (Cochrane, 2008, Hoffman et al., 1985, Kanev and Bierbrauer, 1995). Additional injury to the spinal cord may be caused by a water hammer effect (whereby the pulsatile cerebral spinal fluid within the spinal cord is met with resistance from the mass exerted by the lipoma and has the potential to cause neurological damage), and from intense physical activity (Kanev and Bierbrauer, 1995). When considering treatment options and potential surgical outcomes, it is important to differentiate between tethering due to a mechanical cause which may be reversible with that of the dysplasic cord, which is intrinsic and fixed, and therefore not amenable to improvement from surgical intervention.

Yamada and colleagues (Yamada et al., 1995) found from their research in both human and animal studies, that stretching / tethering of the spinal cord resulted in ischaemia, with impaired oxygenation and metabolism, and deranged electrical activity in the interneurons. The authors suggest that Magnetic Resonance Imaging scans (MRI) may not always provide a complete picture of the spinal cord with regard to tethering and that an understanding of the associated pathophysiology may assist surgeons in diagnosing and managing patients with suspected tethered cord. In a more recent study by Yamada and colleagues (Yamada et al., 2007), 57 patients with tethered cord were monitored using reflection spectrophotometry to record changes in reduction / oxygenation (redox) of the mitochondrial cytochrome using Reflection Spectrophotometry; this optical technique obtains a continuous signal of intracellular metabolism using two light beams of different wavelengths, to provide information of blood volume and oxygenation usage. The patients were grouped into three groups according to the severity of their tethered cord syndrome, and the spinal cords of all patients were subjected to short periods of neuronal stimulation or hypoxic stress to determine the basic redox state of the mitochondrial cytochrome. The patients underwent spinal cord untethering and the redox state of their mitochondrial cytochrome measured again. The results showed increased oxygenation of the mitochondrial cytochrome following untethering of the spinal cord, and this correlated with improved neurological

improvement in all patients, with those with more severe tethering pre operatively displaying reduced oxygenation of the mitochondrial cytochrome and a slower post-operative recovery compared to those with less severe symptoms of tethering. These data support the concept that changes in blood flow correspond to tethering and untethering of the spinal cord and resulting changes in neurological function. The authors acknowledge the need for future research and suggest that further diagnostic studies should include vivo reflection spectrophotometry to examine if the cytochrome oxidase level is reduced in the spinal cord mitochondria. In addition, Yamada and colleagues suggest further studies include histology of the resected tissue, and measurement of anal sphincter contraction (Yamada et al., 2007).

A literature review of tethered cord undertaken by Stetler and colleagues (Stetler et al., 2010) identified 4 papers describing the pathophysiology of tethered cord. Pang and Wilberger (Pang and Wilberger, 1982) studied 23 adults with tethered cord and concluded that repeated “tugging” of the lower end of the cord in adulthood with associated traction on the cord precipitated the onset of tethered cord. Schneider and colleagues (Schneider et al., 1993) studied 10 children with tethered cord using in vivo laser Doppler flowmetry; the results demonstrated a decrease in blood flow in the spinal cord, which normalized to control levels following surgical cord release, and correlated with improved neurology. The authors of the literature review challenge the earlier studies by Yamada and colleagues, suggesting that the reduction in cytochrome redox state could be caused by experimental reduction/ loss of blood supply and that the cause of hypoxia in tethered cord could not be proved by Yamada’s experiments.

Stetler and colleagues conclude that tension on the spinal cord from tethering reduces the normal blood flow and that as this tension accumulates over time (i.e. as the child grows and the spinal cord lengthens), the cord becomes ischaemic with associated clinical deterioration (Stetler et al., 2010).

1.9.2. Radiological diagnosis of LSL and indicators of potential deterioration

Although Magnetic Resonance Imaging scans (MRI) provides the optimum tool by which to diagnose LSL, ultrasound can provide good imaging of the terminal spinal cord in the infant under three months of age and provide a preliminary diagnosis, which may / may not prompt the need for more detailed imaging (Thompson, 2010b).

Although MRI is limited in its ability to detect tethering of cord, it remains the optimum tool by which to image the normal cord or the cord with a spinal lipoma. Anatomical detail provided by MRI is valuable for diagnosis and surgical planning, and until recently the appearance of the cord and lipoma was not thought to differentiate between those at risk of deterioration and those who are not (Dorward et al., 2002). More recently however, a study by Wykes and colleagues (Wykes et al., 2012) has suggested that lipoma type can predict the likelihood of deterioration, with an increased rate of deterioration in transitional lipomas, whereby the spinal cord rotates causing asymmetry, forward displacement and shortening of the nerve roots, in particular those supplying sphincter innervation.

In addition, further prognosis regarding outcomes can be provided by MRI by highlighting the presence of a terminal syrinx (cystic dilatation of the lower end of the spinal cord) or syringomyelia (central cavitation of the spinal cord) and irrespective of the anatomical location of the lipoma, these children have been shown to deteriorate more rapidly than those without (Gupta et al., 2006, Xenos et al., 2000b, Wykes et al., 2012). The pathophysiology, clinical features and optimum surgical treatments are not clear as it is difficult to separate symptoms and signs attributed to syrinx alone, rather than cord retethering (Koyanagi et al., 1997a). However, the presence of a syrinx in association with LSL and in particular a transitional LSL is a poor prognostic feature and further research is needed to more fully understand the relationship between the two (Wykes et al., 2012).

Tethered spinal cord remains challenging to diagnose by MRI scan and diagnosis remains foremost a clinical one (McLone D, 2001).

1.9.3. Symptoms of clinical deterioration

There are four main clinical symptoms associated with deterioration and these are described below:

1.9.3.1. Neurology including pain

Neurological evaluation involves assessment of muscle power, strength, tone, reflexes and sensation including pain.

Clinical examination including reflexes will highlight any deterioration in the child's neurology. Table 1.1 shows lumbar sacral nerve and muscle innervation.

Table 1.1 Lumbar sacral nerve and muscle innervation

Nerve	Movement	Muscle
L2 (L1-L3)	**Hip flexion**	**Iliopsoas**
L3 (L3, 4)	**Knee extension**	**Quadriceps**
L4, 5	**Ankle dorsiflexion**	**Tibialis anterior**
S1	**Ankle plantarflexion**	**Gastrocnemius**
S2	**Great toe abduction**	**Abductor hallucisbrevis**
S3, 4	**Sphincter contraction**	**Deep perineal muscles**

A reflex is a rapid, involuntary response to a stimulus and is made in conjunction with the child's muscle tone, muscle strength and medical history to provide a picture of the child's neurological status and can be used to identify the following:

- Nerve roots: damage to nerve roots can result in depressed reflexes in line with motor and sensory deficits.
- Peripheral nerves: damage to peripheral nerves can result in depressed reflexes that exceed muscle weakness.

- Muscles and neuromuscular junction: damage / deficits can result in depressed reflexes that equate to loss of strength, leaving the child unable to move forcefully.

The two reflexes pertinent to children with LSL are the patella reflex, which assesses any deficits in the quadriceps muscle, patella tendon or nerve root L3 and L4; and the Achilles reflex, which assesses any deficits in the Gastrocnemius muscle, Achilles tendon or nerve root S1.

Deep tendon reflexes are graded as follows:

- 0 = no response; always abnormal
- 1+ = a slight but definitely present response; may or may not be normal
- 2+ = a brisk response; normal
- 3+ = a very brisk response; may or may not be normal
- 4+ = a tap elicits a repeating reflex (clonus); always abnormal

(Thompson, 2012)

An assessment of muscle strength and tone is correlated with function in determining the level of impairment including deterioration. The Medical Research Council provides a validated scale for muscle strength (MRC, 1981) and serial muscle charting provides an important assessment tool in children with LSL.

Long tract signs involving the corticospinal tracts are often variable in children with LSL who may present with a combination of upper and lower motor neuron findings, for example brisk knee reflexes (L3/L4) and absent ankle reflexes (S1/S2). This reflects the pathology located at the junction of the terminal spinal cord and the peripheral nerves. Hyperreflexia, sensory loss of lower limbs and deteriorating motor function may also occur. Children with transitional / chaotic lipomas often have rotation of the spinal cord and this may result in asymmetrical clinical presentation in terms of motor, sensory and orthopaedic deficits (Pang et al., 2009).

Neurological symptoms associated with LSL are often embraced under the term neuro-orthopaedic syndrome or tethered cord and can include gait disturbance, motor, sensory, myelopathic changes and pain (Pang et al., 2010).

Walking and balance change with motor development, with children using different walking strategies throughout childhood. Mobility is dependent on motor development, central nervous system development, gender, culture, physical activity and the presence and type of lower limb joint and / or skeletal deformity (Bala et al., 2009). Even subtle differences in balance and mobility can have an impact on schooling functioning and HRQL (Eiser and Morse, 2001) and assessment appropriate to the individual child will help guide holistic management.

1.9.3.1.1. Pain

Asymmetrical tethering of the lipoma produces a torsional effect on the nerve roots on the superior side compared to the shorter nerve roots on the inferior side of the cord, thus accentuating to the process of tethering and rotation. This rotation of the terminal spinal cord and differential lengths of distal nerves is responsible for the frequently encountered asymmetry of the distal neuro-orthopaedic findings. Neuropathic pain can occur as a result of direct nerve root damage or compression, ischemia, or inflammation. Jensen and colleagues suggest that one cause of neuropathic pain is due to neuronal hyper excitability in neurons that have lost their normal patterned input (Jensen et al., 2011). The pattern and epidemiology of neuropathic pain differs between children and adults, alterations in neuronal plasticity and reorganisation in the young child may contribute to children being less likely to experience neuropathic pain following peripheral nerve damage (Walco et al., 2010). Pain may also be due to underlying nociceptive mechanisms such as musculoskeletal pain and this can be a result of asymmetrical cord tethering with resulting muscle and joint deformities. Musculoskeletal pain can be acute or chronic, diffuse or focal and can result in weakness, stiffness, limited motion, peripheral nerve irritation and widespread and persistent pain. Symptoms may increase with greater tissue injury and inflammation, with the release of inflammatory mediators activating

and sensitising nociceptors resulting in an increased response and decreased threshold (International Association for the Study of Pain, 2010). Musculoskeletal pain is exacerbated by physical activity with potential avoidance of that activity.

In the younger child, pain in children with LSL including tethered cord is frequently described as non-specific, with pain being poorly localised (McLone, 2001). The occurrence or increase in back and leg pain on exertion in the older child however is recognised as one of the important factors in heralding the potential occurrence of tethered / retethered cord (Thompson, 2010a) and requires standardised assessment methods on which to base clinical decision making and intervention.

Some symptoms respond better to spinal cord untethering than others: Pain is the symptom which has been found to respond most favourably to surgical intervention, whereas bladder function may exhibit only partial resolution with no change from the pre-operative state (Colak et al., 1998). Most authors agree that neurological function once lost is unlikely to be regained and that even in the unoperated asymptomatic patient, progressive neurological and urological deterioration, orthopaedic deformities and pain are likely to occur (Hoffman et al., 1985, Satar et al., 1997, Kanev et al., 1989, Xenos et al., 2000b).

1.9.3.2. Orthopaedic

Musculo-skeletal deformities associated with LSL are present in one third to one half of children at initial presentation, most commonly congenital talipes equinovarus (Pierre-Kahn et al., 1997). Imbalance between agonist and antagonist muscle groups predisposes the child to joint deformity as the child grows and the resulting abnormal muscular-skeletal physiology can result in asymmetrical leg length, cavovarus, cavus, and equinocavovarus, hip dislocation and progressive scoliosis, the latter of which can be improved or stabilised following untethering of the spinal cord (Herman et al., 1993, La Marca et al., 1997, Pierre-Kahn et al., 1997). Foot deformities may be present at birth and progress in severity with growth, and soft tissue procedures are

sometimes required for correction of cavovarus, cavus, and equinocavovarus in the younger child, with orthopaedic procedures occasionally required in the older child (Gourineni et al., 2009).

In addition to leg length discrepancies, asymmetric muscle wasting in the calves or buttocks may occur and symptoms of numbness or painless skin lesions may also be present. Changes in orthopaedic status need to be considered in the overall picture when assessing neurology and the term neuro-orthopaedic syndrome is often used to describe the combined neurology and orthopaedic status of patients with LSL.

1.9.3.3. Neurogenic bladder

Urination in a healthy infant is thought to be a spinal reflex action with voiding controlled by the parasympathetic nervous system and triggered by sacral nerves 2 to 4. The detrusor muscles of the bladder wall contract and the urethral sphincter that is also under the control of the pudental nerve relaxes, with resulting micturition. Inhibition of voiding is controlled by the sympathetic nervous system and controlled by thoracic nerves 11 to lumbar nerve 2; detrusor contraction is inhibited, thus avoiding micturition.

As a healthy child grows, bladder storage capacity increases, control over the external urethral sphincter matures and cognitive control over the inhibition or initiation of micturition develops (Neveus and Sillen, 2012). Maturation of the detrusor and sphincter co-ordination varies between infants of one to two years old, with voiding pressures being higher than in adults, with intermittent voiding patterns (Yeung et al., 1995).

Dysgenesis of the lumbar sacral spine and nerve roots in children with LSL may result in impaired innervation to, and function of the lower urinary tract, with resulting bladder dysfunction known as a neurogenic bladder. Abnormal detrusor activity can occur in children with LSL, with contraction of the detrusor muscles of the bladder simultaneous to contraction of the urethral sphincters (detrusor-sphincter dyssynergia) resulting in obstruction of urinary flow, and

increased detrusor contractile activity (detrusor hyperreflexia) resulting in urinary incontinence or inconsistent voiding patterns.

A history of urinary tract infections in an infant may indicate incomplete bladder emptying and be suggestive of abnormal detrusor and sphincter function, and an increase in the number of infections may indicate deterioration in overall urinary function. Deterioration can be further identified by comparing the child's previous urodynamic studies to their current ones, in line with guidance from The International Children's Continence Society, which recommends the use of ultrasound, nappy alarms and uroflometry as age appropriate (Bauer, 2011). It is important to recognise the abnormal but stable status of bladder function in children with LSL verses a deterioration, which often heralds the presence of tethered cord (Rendeli et al., 2007). Regular standardised urodynamic assessment is therefore an essential part of the management of children with LSL to make the crucial functional distinction between a high-pressure bladder (unsafe) and a low pressure one (safe), to maintain healthy renal function and to monitor for deterioration.

1.9.3.4. Neurogenic bowel

Bowel control is acquired in the healthy child by approximately four years of age, with bowel maturation and continence usually preceding urinary continence (Koff, 1988). As sacral nerves 2-5-control bowel function, lack of nerve innervation due to the presence of a LSL can result in abnormal peristalsis with slow gut motility and resulting constipation and / or faecal incontinence. Reduced / lack of innervation between the cerebral cortex and the lower spine can also result in a lack of awareness that the rectum is full of stool, an inability to empty the rectum completely, a failure of the anal sphincters to remain closed and resulting faecal incontinence / soiling. The presence of constipation may cause bladder detrusor instability with resulting urinary retention, incontinence and urinary tract infections (Hellerstein and Linebarger, 2003). Constipation will result in a bowel full of stool and this faecal load presses on the bladder and urethra, further disrupting bladder emptying. Therefore, in managing children with neurogenic bowel and bladder (and this may be present in varying degrees

in children with LSL) both issues must be treated simultaneously. Some children have a fixed level of neurogenic bowel function and deterioration on its own is uncommon in children with LSL.

Dysfunctional innervation to the bladder and bowel secondary to the presence of a LSL requires long term surveillance and management, with lipomas with more complex pathoembryology (e.g. transitional and chaotic lipomas) more likely to have more severe symptoms (Kanev and Bierbrauer, 1995, Pang et al., 2010).

1.10. The importance of standardising clinical assessment tools in identifying outcomes.

It is important to stratify the difference between the asymptomatic children who warrants periodic surveillance, from the symptomatic child who if stable, requires regular surveillance and if progressive, requires surgery for spinal cord untethering and lipoma resection. If deterioration is identified, the plan is to stabilise the child's condition by surgical intervention where appropriate, with the aim of preventing further deterioration. However, detecting clinical deterioration in its earliest stages is difficult, particularly in bladder function where assessment is challenging in the pre continent child. Input is required from not only multiple health care professionals but also from the patients, although many may still be too young to contribute detailed symptoms. This makes it all the more essential that methods of assessing children with LSL are kept objective and standardised in order to allow comparisons to be made, not only of individual patients over many years, but also between series of patients, who may be undergoing different management strategies. Current methods of assessment in the clinic setting are limited by several factors and these include the potential variability in assessment methods between clinicians, differing pathways and protocols for management of this diverse disease and time commitments in a busy clinic setting. Initiatives have been commenced by NHS England (the operating name of the NHS Commissioning Board) to start to address such inconsistences, report outcomes in health care and improve the health of the Nation, and this is discussed below.

1.10.1. The effect of LSL type on outcomes

Linking clinical symptoms with specific pathology or specific LSL type may enable the clinician to offer the family a potential prediction regarding longer-term outcomes for their child and to identify potential future resource requirements. The literature suggests that children with LSL who have a syrinx for example, have a worse prognostic outlook than those without in terms of neurological and urological function (Xenos et al., 2000b, Wykes et al., 2012).

The natural history of LSL varies across individual patients with Dorward suggesting that one end of the spectrum consists predominantly of boys presenting with skin stigmata who are neurologically and urologically stable, to the other end of the spectrum consisting of predominantly girls, with large sacral lipomas and neurological and urological dysfunction (Dorward et al., 2002).

Further associations between lipoma type and potential predictors of outcomes are examined in the systematic review and form a research aim for the thesis.

1.10.2. National Health Service England and policy development

1.10.2.1. The need for change

Most paediatric neurosurgical conditions are rare and require a resource intensive infrastructure for delivery of high quality care and this needs to be balanced with the need for local provision of adequate emergency care including trauma and hydrocephalus management. Government initiatives have been implemented in response to the recognition of the need for safe and standardised practice. One of the aims of The National Health Service (NHS) England is to improve the health outcomes for paediatric neurosurgical children across the NHS (NHS England, 2015). Prior to this, there were no nationally accepted standards or standardised care for paediatric neurosurgery, with concerns regarding variable outcomes specifically in morbidity and mortality for paediatric trauma, tumour outcomes and for management of the hydrocephalic child. These concerns led to a review of paediatric neurosurgical services that

resulted in NHS commissioners producing Safe and Sustainable paediatric neurosurgical service specifications, standards and models of care (NHS England, 2013/14). Children's Neuroscience Networks were established as a result of the review, with an increase in the paediatric neurosurgical consultant workforce to address the requirements of the new Networks. Concerns however continue that this increase may dilute individual practice in subspecialist paediatrics and compromise quality of care.

Guidelines have been introduced to provide transparency of practice and standardisation of care including outcomes:

1.10.2.2. Care pathways

A parent and a group of multidisciplinary clinicians from across the country produced 5 models of care pathways, which included epilepsy surgery hydrocephalus, brain and spinal tumours, brain trauma and spinal dysraphism. Fundamental to care pathways is the need to have a tangible measure of outcome. A general term of mortality for example is a poor discriminator and too restrictive for measuring outcomes of head injury and hydrocephalus. The NHS Specialised Services standards document "Children's Neuroscience Network (for the Neurosurgical Child) Specification Standards" published in 2012, describes the care pathways in more detail (NHS England, 2013/14).

Specific to spinal dysraphism, the commissioners proposed the publication of surgical outcome results with regard to mobility status and sphincter continence at 1 and 5 years post-surgical intervention. The commissioners require a multidisciplinary approach to the child's care, specific guidelines to assess and monitor the upper and lower renal tracts of this group of children and standardised physiotherapy assessments measuring muscle strength before and following intervention.

1.10.2.3. Paediatric neurosurgical Operational Delivery Networks (ODNs) and Clinical Reference Groups (CRG)

Paediatric neurosurgical Operational Delivery Networks (ODNs) and Clinical Reference Groups (CRG) were set up to take these models of care into practice. ODNs are determined by patient need with the aim of assisting the coordination of patient pathways between providers and ensuring specialist expertise and the provision of resources for all patients. ODNs have been established in adult critical care, neonatal critical care, major trauma and burns care, with paediatric neurosciences ODNs currently being established. The work for specialised services is structured around the CRGs, which comprise of a group of clinicians, commissioners, patients, carers and public health experts. These groups use their expert knowledge to advise NHS England on the development of commissioning, service specifications, improving quality and identifying innovation.

The effectiveness of these measures is to be audited through published outcome data, with the aim of reducing variations in practice (Young, 2014).

1.10.2.4. Current neurosurgical audit

The Neurosurgical National Audit Programme (NNAP) has been established by the Society of British Neurological Surgeons (SBNS., 2013). Robust databases, audits and registries are in progress but currently limited to operative mortality with the aim to broaden outcome measures in the future. Individual units will be required to provide reliable data, which can then be published, thus promoting and encouraging improvements in morbidity and mortality within neurosurgery. Consultant Outcome Publication was implemented in 2014, to further encourage transparency and sharing of surgical outcomes.

The British Paediatric Neurosurgery group (BPNG) is affiliated to the SBNS and collects data annually from all paediatric neurosurgery units in the UK and since 2008, data from over 20,000 operations has been collected. The audit is currently limited to operative numbers but the aim is to develop an audit tool to

identify outcomes specific to paediatric neurosurgery, which will allow transparency and promote standardisation of outcomes.

Both the SBNS and the BPNG aim to reflect the distribution of national neurosurgical activity as a step towards the provision of more specific audit and outcomes, both within adult and paediatric neurosurgery. It is envisaged that the sharing and standardisation of practice will assist in improved outcomes for patients.

Audit data can also be obtained from Benchmarking. Benchmarking practice as identified by NHS England aims to identify and provide best practice and care provision and this can influence clinical care, service provision and redesign. The NHS Benchmarking Network National Audit for Intermediate Care (NHS Benchmarking Network, 2014) has been supported by the commissioners as it has provided benchmarked information on activity, outcomes and service models. Although benchmarking has not been implemented in neurosurgery, The National Paediatric Neuroscience Nursing Benchmarking Group (www.pnbg.org.uk) was established in the UK in 2000, with the aim of assessing evidence based care and providing consistent nursing care, to neurosurgical children. The benchmarking principles of the Pnbg are aligned to the principles identified by NHS England in providing optimum and consistent standards of care to children undergoing neurosurgery. Specific to spinal dysraphism, the group produced a validated spinal observation chart, to accompany the group's adaptation of the Glasgow Coma Chart and provides audit and consistency in practice.

1.10.2.5. The future

Data continues to be collected from the SBNS and the BPNG, and benchmarking work continues from the pnbg, the latter which aims to provide evidence based changes in nursing practice.

Additional measures of outcome provided by NHS England include the use of the Commissioning for Quality and Innovation scheme (CQUIN) (NHS 2013-14) and Patient Related Outcome Measures (PROMs) (NHS 2015).

The CQUIN framework acts as an incentive scheme between a commissioner and a provider with the aim of promoting quality of care by linking specific successful outcomes to the provision of increased money to the provider. There are a number of nationally mandated CQUINs, the most embedded being the Friends and Family Test by which patients and relatives provide feedback of their hospital experience, and the NHS Safety Thermometer, by which data is collected regarding falls, urine infections, pressure ulcers and deep vein thrombosis.

Birmingham children's hospital have implemented CQUINs specific to neurosurgery which are linked to outcomes following shunt surgery for management of hydrocephalus; Alderhey children's hospital have introduced a CQUIN to measure the outcome of their intraoperative MRI scanner. As yet there are no CQUINS attached specifically to the management of children with spinal dysraphism but there are CQUINS attached to the management of adult spinal surgery that could perhaps be adapted in the future.

PROMs provides a measure of Health Related Quality of Life (HRQL) and health status as provided by the patient. They can be used to measure the impact of care and to identify and address inequalities in resources and health care, thus ensuring that practice is based around what matters most to the patient. The use of PROMs has yet to be integrated into paediatric neurosurgery practice but has been used in adult spinal patients, known as SPINE TANGO and is used to derive data on Patient Reported Outcome Measures (PROMs). Scoliosis, kyphosis and disc surgery are among the conditions assessed and questionnaires are completed to include information regarding surgery, patient follow up and patient self-assessment. SPINE TANGO has become an international spine registry and includes data regarding all complications that occur after discharge and their consequences (Zweig et al., 2009).

Measuring the standardisation of outcomes is challenging in paediatric neurosurgery; reporting of early shunt infection in hydrocephalic children for example does not include the child's gestation, although it is recognised that premature infants are more susceptible to infection (Spader et al., 2015). In 2013, NHS England approved the introduction of quality dashboards with the aim of collecting outcome data on which to provide assurance on the quality of care and reflect NHS Outcome Framework measures and NICE Quality Standards.

All the above initiatives are aimed at standardising and sharing practice within paediatric neurosurgery and this includes the management of children with spinal dysraphism including LSL. The small numbers of children with LSL make it more difficult to accurately reflect outcomes and transparency is required through the publication of specific outcomes, allowing the sharing and comparison of clinical practice.

1.11. Health Related Quality of Life (HRQL)

The phrase "Quality of Life" was first defined after World War 11, when it was used to emphasise that there was more than just material affluence that was important in life (Campbell, 1976). Today, identifying contributory factors when assessing Quality of Life (QOL) can remain a challenge, the concept elusive and interpretation difficult (Karimi and Brazier, 2016).

QOL has been described as an evaluation of an individual's wellbeing and has been defined by the World Health Organisation as "the individual's perception of their position in life in the context of their culture and value systems in which they live, and in relation to their goals, expectation, standards and concerns"(WHOQOL, 1993). There are numerous potential confounding factors in assessing QOL including gender, culture, age, socio economic status and spirituality and QOL can only be understood from the perspective of the individual person.

Many authors describe HRQL and definitions include how well an individual functions in life, how they perceive themselves in terms of health, how health impacts on their life and how they view the value of their health (the loss of QOL due to ill health) (Hays and Reeve, 2010, Karimi and Brazier, 2016).

Health status describes the impact of functional status and the inability to participate in daily activities; the presence of a reduced functional status and the associated environmental and social barriers encountered, can have a negative effect on the QOL / HRQL of children (McDougall et al., 2014). Eiser (Eiser, 1990) found that compared with healthy children, children with chronic disease and physical disability are three times more likely to experience psychiatric illness and are more at risk of social maladjustment; however, those with chronic disease but no physical disability were twice as likely to experience psychiatric illness but were not at risk with regard to social maladjustment.

The terms QOL, HRQL and health status are often used interchangeably (Karimi and Brazier, 2016) and a scale that examines physical aspects for example, may potentially fail to encompass associated psychosocial factors; therefore, the terms QOL, HRQL and health status are used interchangeably in this thesis.

1.11.1. Why measure HRQL?

Measuring HRQL can demonstrate the impact of health on QOL and identify health disparities, and this information can be used to promote equal opportunities and improved interventions for people with disease / disabilities. By measuring HRQL, service delivery and health policy can be improved through the provision of clinical audit and resulting changes in practice. Evaluating HRQL and providing appropriate interventions and support in individual patients and disease specific illnesses can promote Clinical Governance and Evidence Based Care.

HRQL measures help determine how chronic disease and management affect a child, whether treatment is appropriate, effective over time, whether there is a

choice of management or interventions for the child and family, what support might be required and what policy changes might be of benefit (Forsyth et al., 2007). Perception of an individual's HRQL can change over a lifetime (Palermo et al., 2008) and longitudinal studies are important, not only to the individual, but to public health and commissioners, who can plan and provide appropriate timely support and interventions.

Some of the drivers and barriers for measuring HRQL are provided in table 1.2 and 1.3 respectively. The economic factors in understanding HRQL are provide in table 1.4.

Table 1.2 Drivers for measuring HRQL

Drivers for measuring HRQL	
Assessment tool for parent / child	• Improved communication identified between clinician and (Neumann et al., 2012) • Improved understanding of the unique perspective of the parent /child may result in improved outcomes for child (Marino et al., 2009) • Empowered parents through mutual understanding and involvement in care e.g.: PROMS (Patient reported Outcome Measures), some of which provide a measure of HRQL by focusing on the patient's perspective with regard to the effectiveness of interventions, in addition to patient involvement and empowerment (Department of Health and 2008).
Tool to improve service provision	• Identify health disparities with improved interventions and equal opportunities • Improved service delivery and health policy: CQUIN (Commissioning for Quality and innovation scheme), promoting improved response to the individual needs of the patient (NHS, 2013) and a financial incentive for the health care provider for the provision of excellent care.
Evaluation of patient status and the impact of disease and treatment	• Evaluate changes in morbidity • Assess efficacy of medical intervention • Evaluation of progress • Comparison of treatment across individual patients and institutions.

Table 1.3 Barriers to measuring HRQL

Barriers to measuring HRQL	
Perception of validity of qualitative research	• Some clinicians believe assessing the patient's HRQL is irrelevant, unimportant and does not affect treatment, management or outcomes (Espallargues et al., 2000, Fihn et al., 2004).
Response shift	• HRQL self-assessment may alter in line with changing health and the "response shift" highlights the importance of considering when the assessment was undertaken (Eiser and Jenney, 2007).
Limitations	• Specific intervention and treatment planning may be restricted where HRQL measure fails to capture specific, individualised issues (Cuervo et al., 2014).
Financial impact	• Assessing HRQL has financial implications in terms of administrative costs and time.
Time constraints	• Time constraints involve the clinician, child and family.

Table 1.4 Economic factors in measuring HRQL

Economic factors in measuring HRQL	
Economic impact	• Family income can have a detrimental effect on the child's HRQL, but confounding factors include psychosocial factors (Cassedy et al., 2013). • Direct financial cost related to health care and indirect costs due to loss of parental earnings due to the care requirements of the child. • Potential change in public health policy and education to address these points (Witt and DeLeire, 2009).

1.11.2. The child with a chronic illness and HRQL

Chronic illness can have an effect on the child's development as it can change the developmental trajectory; this is particularly relevant during adolescence when changes to social, psychological and physical issues occur (Holmbeck, 2002).

Ravens-Sieberer et al (Ravens-Sieberer et al., 2014) suggest that a child's attitude is dependent on age, development and social factors and these may change as they become more aware of the limitations their illness may place on them in terms of opportunities and future life. The authors highlight the importance of support from friends and peers in the child's ability to adapt to their situation. A study of disabled adolescents found that they placed more emphasis on loyalty, intimacy and friendship groups, particularly with other disabled young people, than they did on their illness (Matheson et al., 2007). However, any disruption to the normal formation of friendships and peer acceptance, for example from the impact of chronic illness, can have an effect on HRQL. A literature review of HRQL of obese children identified that bullying was found to be associated with school absenteeism, social isolation and a reduced HRQL, with psychosocial HRQL inversely related to the frequency of being bullied (Buttitta et al., 2014).

1.11.3. Policy development and HRQL in children with chronic disease

The NHS Outcomes Framework has identified the need to demonstrate that the HRQL for people with long term conditions has been evaluated and appropriate interventions instigated (Department of Health, 2014 / 2015). Furthermore, the framework identifies that individuals with a chronic condition need to feel supported in managing their condition and that their functional ability is improved / optimised. Although not specific to children, the overall framework provides recognition of the importance of assessing the HRQL of individuals with a chronic illness and secondly, highlights the importance of combining health and social care in improving outcomes.

An additional publication entitled “Recommendations to improve the health of children and young people” (Department of Health, 2013) highlights many important issues, including addressing the complications for children with chronic illness such as the co morbidities associated with their condition, the importance of family and friends, and the period of transition to adult services. The document highlights the need for policy development and updating, for strategic planning and the importance of input from the commissioners.

1.11.4. The HRQL of children with LSL

The HRQL of children with spinal lipoma has been minimally explored with only 2 papers identified in the systematic review in chapter 2 of the thesis: A publication from India described children as having a “better social life” post spinal lipoma resection due to an improvement in motor and urinary symptoms (Kasliwal and Mahapatra, 2007); the second study explored the HRQL of children with spinal dysraphism (myelomeningocele and lipomyelomeningocele) as measured by proxy assessment and identified those children with greatest disability (those with myelomeningocele) as having reduced physical and psychosocial wellbeing (Wang et al., 2013).

The HRQL of children with spinabifida aperta (myelomeningocele) has been frequently studied, but the complex spectrum of this group of children presents difficulties when comparing with children with LSL, some of whom may be clinically asymptomatic. However, neuropathic bladder and bowel, pain, orthopaedic and muscular skeletal deficits can occur in both groups, so there is value at looking at the wealth of publications relating to the HRQL of children with spinabifida aperta. A literature review by Sawin and Bellin (Sawin and Bellin, 2010) identified 41 publications with 17 different instruments used to assess the HRQL of children with spinabifida, with one validated instrument, The Health Related Quality of Life SpinaBifida questionnaire (HRQOL-SB) (Parkin et al., 1997) and a second, The Fecal Incontinence and Constipation Quality of Life (FIC QOL) scale (Nanigian et al., 2008) highlighting areas specific to children with spinabifida.

The severity of symptoms in children with spinabifida is hugely variable including the presence of hydrocephalus and a range of neurocognitive abilities/ deficits. This renders the use of assessment tools used specifically for children with spinabifida inappropriate for use with our research group, as many of these factors are not recognised in association with LSL.

The literature regarding children with spinabifida does however highlight the need for an assessment of HRQL to be incorporated into the overall assessment / monitoring of children with LSL and to form part of the management plan with regards to potential physical and psychological interventions and resource requirements.

1.11.5. The International Classification of Functioning, Disability and Health (ICF).

The International Classification of Functioning, Disability and Health (ICF) provides a standardised and validated framework on which to describe the health and health related status of individuals (World Health Organisation, 2001). The ICF classification includes more than 1,400 categories and Aringer and colleagues imply that this complexity limits its use in clinical practice

(Aringer et al., 2006). They suggest that the use of core sets for specific conditions should be designed with input from clinicians and patients worldwide, in order to address the challenges of specific diseases and patient groups.

Early use of the framework suggested that only a small proportion of these 1,400 categories are needed, with approximately 20% of the codes being required in the majority of clinical practice (Ustun et al., 2004). Since using the ICF framework in its entirety is highly time consuming, Raunch and colleagues suggested that a patient's level of functioning should be described using as few core sets as possible to provide a thorough assessment (Rauch et al., 2008).

In 2007, the ICF-CY (Child and Youth Version) was developed to encompass developmental aspects of the child, including play and speech, and can be applied from birth to 17 years old to describe the child's physical, social and psychological development within his / her environment (World Health Organisation, 2007). It provides a multipurpose classification for use in a variety of sectors by a wide range of users. The ICF-CY helps describe changes in body function and structure, the level of capacity of that individual (what they can undertake in a standard environment) and their level of performance (what they actually do in their usual environment). Functioning refers to all physical functions, while disability is an umbrella term to describe limitations to activity, participation restrictions and impairments.

The ICF-CY continues to be developed and shortcomings addressed: In 2012, Cramm and colleagues welcomed the use of the framework in Occupational therapy practice whilst acknowledging that additional clarity was still required regarding activity, participation and functional factors (Cramm et al., 2012).

In 2013, a literature review of 79 papers relating to the use of the framework in physical therapy practice suggested that despite evolving integration of the framework into clinical practice, there remains a need for verifying that the framework actually provides additional value to current models of assessment (Escorpizo and Bemis-Dougherty, 2013).

A literature review exploring the relationship between the ICF-CY and environmental factors in children with motor deficits identified that although physical environmental variables could be linked with categories in the ICF-CY, social environmental variables could not be (Hwang et al., 2014). The authors suggest that assessing social environmental variables is important in understanding and promoting communication between health and social care providers and the child and family, and advise adaptations are undertaken to the ICF-CY framework to encompass these variables.

1.11.5.1. The ICF-CY and children with Spinabifida

Chen and colleagues (Chen, 2007) describe the use of the ICF-CY model for children with spinabifida, elaborating on how core elements of the framework overlap with each. Body structures and functions for example involve reduced lower limb muscle strength and altered sensation, and can pose limitations to activity and participation, within school, to the child's family, his / her social life and to potential employment possibilities in the future. The authors further suggest that participation and limitations to participation (i.e. those activities the child can /cannot undertake at a level appropriate to his / her ability) cannot be seen in isolation, but are linked to personal factors and the environment in which the individual lives. The algorithm in figure 1.12 explains the use of the ICF-CY for children with spinabifida.

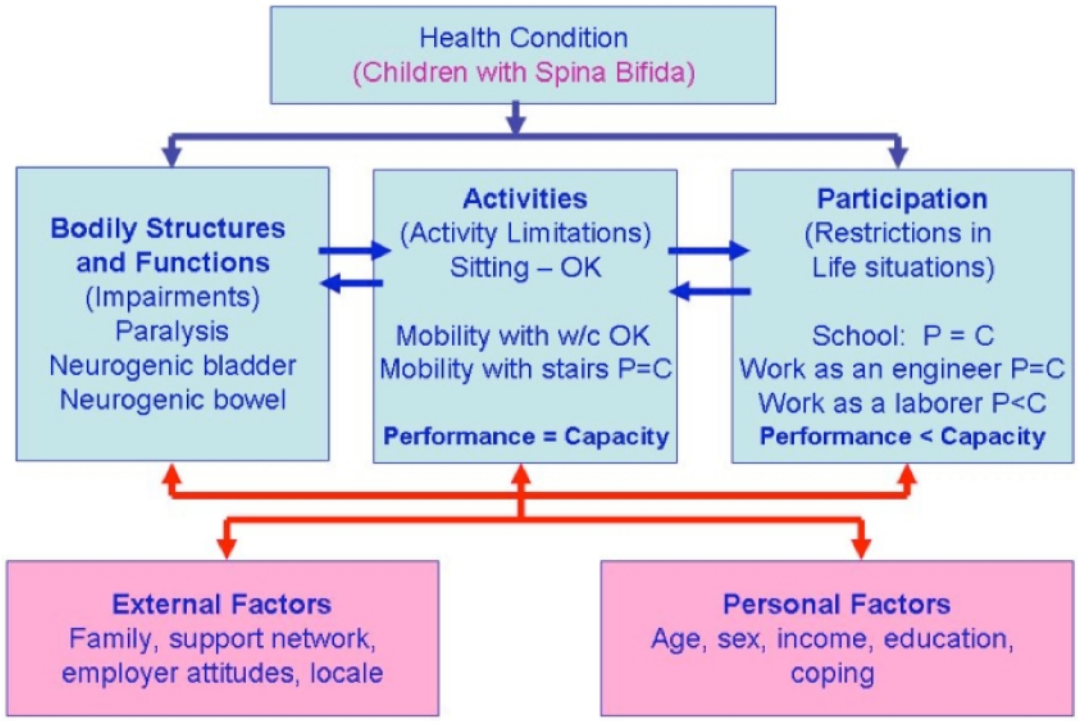


Figure 1.12 The use of the ICF-CY for children with spinabifida
(Chen, 2007)

Most authors agree that in the current framework, the ICF-CY is too cumbersome to use in clinical practice but that with adaptations and refinement, it could prove a useful tool in assessing the child within his/ her own environment. As such, it could be used to assess children with LSL in future studies.

1.12. Self Esteem

Happiness, a sense of well-being and self-esteem are key indicators of HRQL and as such, are important factors in the management of children with chronic disease. Self-esteem describes an individual's personal value or self-worth and encompasses one's own personal beliefs (Trzesniewski et al., 2006). Orth et al (Orth et al., 2014) suggest that low esteem can be driven by one's evaluation of self-worth, as opposed to being driven by specific themes such as physical appearance and peer relationships; several authors suggest low self-esteem

can be a predictive factor of poor mental health including depression in adulthood, with a potential for higher levels of criminal behaviour and reduced financial prospects (Ferro and Boyle, 2013b, Trzesniewski et al., 2006).

The relationship between self-esteem and children with LSL has not been previously assessed. Therefore, a preliminary analysis was undertaken in this thesis to assess if disease specific clinical outcomes correlated with self-esteem and identify if supportive interventions were required.

1.13. Parents of children with a chronic disease

Increased levels of stress, anxiety and depression have been identified in the literature in parents caring for a child with a chronic disease (Cousino and Hazen, 2013). Children's response to illness can be dependent on the attitude and behaviour of their parents and Caicedo (Caicedo, 2014) suggests that parental psychological distress when caring for a child with a chronic disease is a risk factor for poorer mental health for both the child and family members. Increased parental stress has been identified in parents of children with a rare disease, particularly where the disease trajectory is uncertain, as in the case of children with LSL.

An evaluation of parental response to caring for a child with LSL has not been previously undertaken. Therefore, a preliminary analysis was undertaken in this thesis to assess if specific disease specific clinical outcomes correlated with increased parental emotion and secondly, to identify if interventions to improve parental health status and functioning, were required for this group of parents.

1.14. Summary

LSL is a complex congenital anomaly with the potential to cause progressive disability with regard to neurological, urological and orthopaedic function, requiring evaluation throughout childhood and beyond. The complexity and diversity of the pathology of LSL in individual children results in a wide range of symptomology but the rarity of the disorder results in inconsistent methods of

assessing such patients. Clinical assessment remains the optimum method of evaluating and monitoring symptoms in terms of stability, improvement or deterioration, in relation to the developmental milestones of childhood. The literature reflects the challenges in assessing and scoring these symptoms due to the age related changes in mobility, gait, pain assessment, and bladder and bowel function and control.

However, early detection of deterioration is recognised as essential to enable appropriate intervention to attempt to stabilise the child's condition and prevent further deterioration (Wang et al., 2013). The aim of surgical treatment is to untether the spinal cord in order to prevent progressive orthopaedic deformity, neurological damage and to preserve sphincter function. Current controversy surrounds the management of the asymptomatic child: those who advocate surgery on the presumption that prophylactic surgery will prevent the inevitable deterioration (Pierre-Kahn et al., 1997) and those who advocate a conservative policy whilst the child remains asymptomatic (Kulkarni et al., 2004b). Pang and colleagues (Pang et al., 2010) published their operative results of 238 patients with spinal lipoma and states that when compared to published data, those asymptomatic patients who underwent complete resection of the lipoma had better outcomes than asymptomatic patients who had not undergone surgery, and that surgical technique was the most important factor in determining outcome.

Current methods of clinical evaluation are non-validated and are difficult to apply. The requirements of an assessment tool must include ease of use, reproducibility and comparability. Such a tool would enable the clinician to decide if and when to operate and provide a standardised measurement of outcomes following surgery. Longitudinal data would enable him / her to compare data across international neurosurgical units and improve care by comparing treatment and outcomes.

LSL is a lifelong disease, which changes throughout childhood. Chronic disease is associated with a reduced HRQL and this has been highlighted in children with other types of spinal dysraphism including spinabifida (Rendeli et

al., 2007). NHS England has highlighted the importance of assessing HRQL in people with chronic disease, not only with the aim of providing interventions where appropriate, but ensuring policies are in place to provide equality of required service provision.

This chapter has provided an introduction to normal physiology of neural tube closure and the abnormal pathophysiology associated with spinal dysraphism and LSL in children, including deterioration. The importance in standardising assessment, the publication of outcomes and the inclusion of HRQL measures have been highlighted as important as identified by initiatives developed by NHS England (NHS England, 2013/14, NHS England, 2015).

To understand what current objective methods of assessment are used to evaluate children with LSL, a review of the literature was undertaken and is presented in the following chapter.

Chapter 2. A Systematic Review of the symptomology associated with lumbosacral lipoma (LSL) in children and the tools used to assess them.

2.1. Aims and scope

In chapter one the anatomy, physiology and symptomology associated with lumbosacral lipomas (LSL) in children were discussed. A previous systematic review of lipomyelomeningocele (LMMC) to assess the reported symptoms and assessment tools being utilised nationally and internationally, identified a lack of consensus about terminology regarding the condition (May et al., 2013). Furthermore, this review only covered a proportion of children with LSL and as a consequence, a second review was undertaken to encompass LSL specifically. Studies involving a mixed group of adult and paediatric patients and of mixed etiopathophysiology were also included. Out of 14,833 papers identified, 54 papers were identified for full review. The methodology and findings of the review are provided in this chapter.

The need for regular assessment and monitoring of this group of children is well-recognised and is important as clinical deterioration is often irreversible: evidence from several large series suggests that once neurological function has deteriorated it is unlikely to be regained, and that even in the untreated asymptomatic patient, progressive neurological and urological decline, pain and orthopaedic deformities are likely to occur (Byrne et al., 1995, Koyanagi et al., 2008, Satar et al., 1997, Xenos et al., 2000b, McCairn and Jones, 2014). Initial evaluation and ongoing surveillance of children with LSL requires objective, consistent methods of assessment to identify those children with early clinical signs of deterioration and tethered cord, including back and leg pain, neurological and orthopaedic deterioration and deterioration in sphincter function, in particular the bladder. The need for assessment tools by which to monitor this group of children led to the systematic review in order to identify what is already being utilised.

One important element of assessment is Quality of Life. Quality of Life (QOL) has been described as an evaluation of an individual's wellbeing (Freeman et al., 2013) and has been defined by the World Health Organization as "the individual's perception of their position in life in the context of their culture and value systems in which they live, and in relation to their goals, expectation, standards and concerns" (WHOQOL, 1993). Health is not seen as merely the absence of disease and confounding constructs including functional status, health status and disease trajectory must be recognised as influencing, but not defining, QOL. Eiser states that "over - reliance on physiological indices neglects the important question of how illness or treatment impacts on children's own ratings of their health or well-being" (Eiser, 2007). Health Related Quality of Life (HRQL) refers specifically to the impact that both health and illness have on Quality of Life (QOL) and this encompasses the impact that a disease or medical treatment has on the individual's ability to experience satisfaction (Drotar, 2004). In addition, measuring the HRQL in children with chronic conditions is important in evaluating the efficacy of interventions and changes in morbidity. As LSL is a chronic illness spanning the child's lifespan, assessment tools by which to measure HRQL were recognised as important and thus included in the review.

The current systematic review sought to identify what symptoms are assessed and how these are measured in children with LSL. The methodology and results of the review are presented and recommendations for future research suggested.

2.2. Methodology

The Centre for Reviews and Dissemination (CRD) in York (University of York, 2009) provides guidance by which to undertake a systematic review and is used to provide a framework for the current systematic review.

The acronym PICO (Population, Intervention, Comparison, Outcomes) can be used to help define a review question and all, or components of the PICO, can be selected to guide the review process. By helping define a clinical question,

the PICO framework ensures comprehensive search strategies are undertaken (Haroon and Phillips, 2010), guides the researcher to relevant literature and helps identify research gaps or inconsistencies in the review process, which can then be addressed in future studies (Robinson et al., 2011).

The adapted PICO for this systematic review is as follows:

P: The population comprised children from birth to eighteen years of age diagnosed with LSL, and their families. Due to the heterogeneity of the condition, papers including both children and adult patients were included.

The letter “I” depicting “Intervention” and “C” depicting “comparisons” in the PICO framework were omitted in the review process as there were no interventions or comparisons.

O: Outcomes comprised parameters of physical and psychological functioning and the tools by which these parameters were assessed.

Use of the adapted PICO framework resulted in the following question for this systematic review:

What is the symptomology associated with lumbosacral lipoma (LSL) in children and what tools are used to assess the symptoms?

2.2.1. Types of studies

All studies other than case studies were included in the review if they discussed the symptoms associated with LSL in children including descriptive studies, as these studies provided an understanding of the heterogeneity of the condition and consequent diversity of assessment tools. Case studies were omitted, as they were not considered to contain the level of detail required to assess this rare anomaly.

2.2.2. Types of participants

Although paediatric patients include children and young people defined by age, the optimum age of transition of patients to an adult centre in the UK is still variable (Lewis and Smith, 2010). Due to the rarity of LSL, adult and paediatric patients were included in the review, with papers relating specifically to adult patients being excluded.

2.2.3. Types of outcomes

A variety of assessment tools are used to diagnose the symptoms associated with LSL and resulting tethered spinal cord. MRI scans can be used to identify the presence of LSL and any associated spinal syrinx, spinal cord movement (or lack of it) associated with tethered cord (Blount and Elton, 2001) and to identify cerebrospinal fluid (CSF) movement and blood flow associated with tethered cord and resulting ischemic changes (Brunelle et al., 1996). However, although anatomical detail provided by MRI is valuable for diagnosis and surgical planning, the appearance of the spinal cord and lipoma does not consistently differentiate those at risk of deterioration from those who are not: MRI scan appearances have been shown to be no different in cases where retethering is clinically evident, to those patients who have undergone successful surgical untethering (Colak et al., 1998).

Therefore, despite technological advances the diagnosis of tethered cord secondary to LSL remains foremost a clinical one (Dorward et al., 2002). A robust system of assessing, monitoring and recording the child's clinical status is essential in early detection of deterioration and to determine subsequent management. It is therefore necessary to try and identify which clinical parameters might form the basis for that system.

The methods of assessing symptomology in children with LSL include outcomes relating to neuro-orthopaedic syndrome (encompassing neurology, orthopaedic factors and pain) with outcomes / symptoms often described in terms of stable, improved or deteriorated. Deterioration in urological function often heralds the

appearance of a tethered cord and thus publications relating specifically to urodynamic function in children with LSL were included, the majority of such publications also address neurogenic bowel.

Varni and colleagues (Varni et al., 2007a) assessed the HRQL of 2,500 children with chronic disease across 33 different disease categories, and compared them with a group of 9,500 healthy children using the validated Paediatric Quality of Life Inventory (PedsQL) (Varni et al., 1999). The authors identified that the children with chronic disease had an impaired HRQL compared with the normative values and that there were substantive impairments in emotional, social, physical and school functioning domains. They further concluded that their findings suggest clinical implications for healthcare providers including an urgent need for targeted treatment for children with chronic disease.

Danielsson (Danielsson et al., 2008) studied 38 children with spinabifida to identify what factors effected the HRQL of this group of children with regard to their function and ambulation. He concluded that reduced functional mobility was associated with a reduced physical HQQL compared to healthy individuals. The relationship between the level of functional mobility and HRQL is further confirmed in a study by Wang (Wang et al., 2013) who studied 32 children with myelomeningocele and 28 children with spinal lipoma. He found the children with more severe physical disability (those with myelomeningocele) had a reduced HRQL than those with less physical disability (those with spinal lipoma). Children with spinal lipoma had reduced scores of HRQL in some areas but the author suggests that that an increase in the number and severity of medical problems in either group was associated with a reduced HRQL.

The range of symptoms including mobility in children with LSL is highly variable and it is important to identify how the disease process and interventions affect the child's HRQL. Chronic disease is associated with increased morbidity including a reduced HRQL (Varni et al., 2007a) and therefore HRQL was identified as an important assessment tool to include in the current systematic review. Eiser and Morse (Eiser and Morse, 2001) suggest that a deterioration in health may result in exclusion from activities with a consequent reduction in

self- esteem in addition to a reduced HRQL. Therefore, self-esteem was included in the search strategy.

In addition to identifying what tools are used to measure the HRQL in children with LSL, it is important to identify any inconsistencies or gaps in the literature regarding HRQL measures and children with LSL.

2.3. Search Strategy

An initial search of the Cochrane Library was undertaken (http: www: cochrane.org) in line with the Systematic Review and Guidance provided by the 2009 NHS Centre for Reviews and Disseminations (University of York, 2009). The search did not identify any such reviews for children with LSL. A multiple database search strategy was then performed for papers published between January 3rd 1990 and December 3rd 2013. An update of publications was undertaken in March 3rd 2015 to identify any further publications pertinent to the review and no new publications were found.

Prior to 1990 much ambiguity existed regarding LSL although the first detailed description was by Chapman in 1982 (Chapman, 1982). Advances in radiology- in particular MRI, alongside advances in surgical instrumentation and techniques have occurred since. It was therefore deemed appropriate by the two reviewers, to commence the review from 2000.

The search criteria included terms for the population (children and young people), LSL and associated etiologies, assessment tools and HRQL; the search components were combined with the Boolean operators “OR” and “AND”. Qualitative and quantitative descriptive studies were included in the review process.

LSL is an ambiguous term and consequently the search was widened to include spinal dysraphism, and spinabifida occulta, in addition to the terminology outlined in table 2.1. Databases were selected to include medical, nursing and psychology publications likely to be of relevance for this review.

Table 2.1 Search criteria

CINAHL: Cumulative Index to Nursing and Allied Health Literature (EbscoC). Search date 1990-2013; ISI: Institute for Scientism Information (Web of Science). Search date 1990-2013;PsychINFO: International coverage of psychology and related disciplines (Ovid). Search date 1990-2013;Medline. Search date 1990-2013;Embase. Search date 1990-2013;Cochrane Library. Search date 1990-2013.

MeSH (Medical Subject Heading)	Search term	Limit	Databases
Spinal dysraphism **Lipomyelomeningocele** **Spinal / intraspinal lipoma** **Lumbar sacral lipoma** **Lipomyeloschisis** **Leptomyelolipoma** **Lipomyelocele** **Lipomyelocytocele** **Spinabifida Occulta** **Tethered cord**	Pain Sensation Dermatones Motor function Reflexes Neurology Mobility Urodynamics Urology Bowel function Health Related Quality of Life. Self-esteem Orthopaedic Orthotic	Children / young people. Birth- 18 years	Medline CINAHL Embase ISI Web of Science. National Library for Health Cochrane PsycINFO

The search used both text words and MESH (Medical Subject Headings) terms.

In addition to a search of the Cochrane library previously described, the search criteria outlined in table 2.1 was used to search Medline. The following searches were performed:

1. Lipomyelomeningocele or neural tube defects or lipoma or Cystocele or tethered cord or spinabifida occulta or lipomyeloschisis or leptomyelolipoma or spinal lipoma or intraspinal lipoma using synonyms, variations and all the MESH terms.

Subsequently this first search was combined with the following:

1. Pain (2 and 1)
2. Quality of Life or self - esteem or HRQL (1 and 3)
3. Orthopaedic or clubfoot or (1 and 4)
4. Movement and mobility (1 and 5)
5. Sensation and sensory or feedback sensory (1 and 6)
6. Bowel function or intestine function or bowel or fecal incontinence (1 and 7)
7. Urinary tract or urinary bladder or bladder or urine (1 and 8)

All the searches were then limited to children and started from 2000; the review was updated in 2015.

A similar strategy was then applied to the databases listed above using the same search terms for each database and produced a total of 14,833 papers. An update alert was set up for Medline, PsycINFO and Embase. The final search strategy is shown in figure 2.1.

Table 2.2 Final Medline (Ovid) search strategy (search updated: 03.03.2015)

# ▲	Searches	Results	Search Type
1	exp Neural Tube Defects/ ▶	24469	Advanced
2	neural tube defects.mp. [mp=title, abstract, original title, name of substance word, subject heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier] ▶	8789	Advanced
3	Lipomyelomeningocele.mp. [mp=title, abstract, original title, name of substance word, subject heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier] ▶	194	Advanced
4	exp Lipoma/ ▶	10851	Advanced
5	lipoma.mp. [mp=title, abstract, original title, name of substance word, subject heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier] ▶	12848	Advanced
6	exp Cystocele/ or cystocoele.mp. ▶	362	Advanced
7	exp Meningomyelocele/ or lipomyelocystocele.mp. ▶	3396	Advanced
8	tethered cord.mp. ▶	873	Advanced
9	spina bifida occulta.mp. or exp Spina Bifida Occulta/ ▶	2202	Advanced
10	lipomyeloschisis.mp. ▶	10	Advanced
11	leptomyelolipoma.mp. ▶	2	Advanced
12	spinal lipoma.mp. ▶	121	Advanced
13	intraspinal lipoma.mp. ▶	54	Advanced
14	1 or 2 or 3 or 4 or 5 or 6 or 7 or 8 or 9 or 10 or 11 or 12 or 13 ▶	39120	Advanced
15	limit 14 to (yr="1990 -Current" and "all child (0 to 18 years)") ▶	9638	Advanced
16	pain.mp. or exp Pain/ ▶	591087	Advanced
17	15 and 16 ▶	456	Advanced
18	exp "Quality of Life"/ or HEALTH RELATED QUALITY OF LIFE.mp. ▶	133049	Advanced
19	self esteem.mp. or exp Self Concept/ ▶	82521	Advanced
20	HRQL.mp. ▶	2565	Advanced

21	18 or 19 or 20 ▶	209480	Advanced
22	15 and 21 ▶	215	Advanced
23	exp Orthopedics/ or orthopaedics.mp. ▶	18759	Advanced
24	orthopaedic*.mp. ▶	25789	Advanced
25	clubfoot.mp. or exp Clubfoot/ ▶	3667	Advanced
26	club foot.mp. [mp=title, abstract, original title, name of substance word, subject heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier] ▶	533	Advanced
27	23 or 24 or 25 or 26 ▶	41310	Advanced
28	15 and 27 ▶	121	Advanced
29	movement.mp. [mp=title, abstract, original title, name of substance word, subject heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier] ▶	304392	Advanced
30	mobility.mp. ▶	121694	Advanced
31	exp Movement/ph [Physiology] ▶	140123	Advanced
32	29 or 30 or 31 ▶	509312	Advanced
33	15 and 32 ▶	286	Advanced
34	exp Sensation/ or sensation.mp. ▶	262254	Advanced
35	exp Feedback, Sensory/ ▶	1374	Advanced
36	sensory.mp. [mp=title, abstract, original title, name of substance word, subject heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier] ▶	162470	Advanced
37	34 or 35 or 36 ▶	390864	Advanced
38	15 and 37 ▶	277	Advanced
39	bowel function.mp. ▶	2508	Advanced
40	bowel*.mp. [mp=title, abstract, original title, name of substance word, subject heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier] ▶	114117	Advanced
41	intestine function.mp. ▶	50	Advanced
42	Fecal Incontinence/ or faecal incontinence.mp. ▶	8568	Advanced

43	fecal incontinence.mp. [mp=title, abstract, original title, name of substance word, subject heading word, keyword heading word, protocol supplementary concept word, rare disease supplementary concept word, unique identifier] ▶	9266
44	39 or 40 or 41 or 42 or 43 ▶	121962
45	15 and 44 ▶	347
46	urinary tract.mp. or exp Urinary Tract/ ▶	440706
47	urinary bladder.mp. or exp Urinary Bladder/ ▶	108119
48	exp Urine/ or urine.mp. ▶	207865
49	bladder.mp. or exp Urinary Bladder/ ▶	152191
50	46 or 47 or 48 or 49 ▶	694926
51	**15 and 50** ▶	**1004**

The broad search strategy implemented ensured all relevant studies that related in any way to the topic of interest were retrieved. An initial title screen was undertaken due to time and resource constraints and studies that were irrelevant to the review including case studies, and duplications were excluded at this stage. Following the title screen, abstracts were selected for eligibility by two authors (DT and LM) according to selection criteria. Each included paper that fulfilled the inclusion criteria was coded using a data extraction tool (Appendix 2.1) and included key elements regarding LSL pathophysiology, assessment and management. Studies were excluded based on the following criteria: case studies, papers not published in English, spinabifida aperta without lipoma, spinal lipoma associated with other major abnormalities (ambiguous genitalia, cloacal atresia, and associated syndromes). Any differences were resolved through consensus and if required, a third author was asked to review.

Due to the paucity of publications on LSL, publications were included if they presented adult and children's' data together. A spreadsheet was set up to enable both researchers to assess whether the study was to be retained,

following which these full papers were obtained. The search studies were transferred to Endnote thus enabling removal of duplications and facilitation of storage. Reasons for inclusion and exclusion were noted.

Other key elements extracted were the year of publication, patient characteristics including age, gender, clinical / psychological intervention or lack of, length of follow up, use of validated assessment measures and patient outcomes.

2.3.1. Researching other resources

Google Scholar was searched using the advanced scholar search facility to search for the terms spinabifida occulta, spinal lipoma, children and HRQL. The date of the search was 03.12.2013 and the period of the search from January 3rd 2000 to December 3rd 2013; the search was updated in 2015.

Grey literature was also included in the review and describes literature that is not controlled by commercial publishing, but is created by researchers in a variety of fields including government, industry and other organisations. Grey literature also includes conference proceedings and consequently abstracts from the ISPN (International Society of Pediatric Neurosurgery) and the BPNG (British Pediatric Neurosurgical Group) from 2000 to 2015 were included in the review. Other sources of grey literature included in the search were The Agency for Healthcare Research and Quality (US department of Health and Human Service, 2015) , The National Institute for Health and Clinical Excellence (NICE, 2014), the Grey Literature Report (The New York Academy of Medicine, 2015) and Open Grey at http://www.opengrey.eu.

Contacting lead specialists in a small specialist field such as LSL can be useful in identifying on-going work and relevant references (McManus et al., 1998). Therefore, two authors active in the management of children with LSL were contacted, one in Europe and one in the USA.

Relevant book chapters were searched and finally, the UK doctoral thesis database was searched (www.thesis.com).

Database searching is unlikely to reveal all relevant publications and hand searching of publications is extensively time consuming (McManus et al., 1998). However, due to the paucity of relevant literature specific to LSL in children, reference lists of papers were hand searched for potential additional articles and a citation search undertaken using the Web of Science.

Although excluding non-English language studies can introduce bias (Moher et al., 2003), publications from non-English speaking countries are more likely to be published when written in English (Moher et al., 1996). Therefore, publications not written in English were excluded from the review.

2.3.2. Heterogeneity

It was anticipated that clinical heterogeneity would be high in the review but due to the rarity of the condition and the small number of neurosurgical centres reviewing children with LSL, it was decided not to set such tight restrictions that relevant studies might be excluded. Clinical heterogeneity includes the age and physiology of the individual child, the type (if any) of surgical intervention and expertise of the surgeon, the management of neurogenic bladder and bowel function, how outcomes are measured and over what period of time and in which country the study is undertaken.

2.4. Data extraction

Data extraction forms part of the review process and in addition to providing a record of decisions made during the review process, it captures the methodology and study characteristics of studies (Brown et al., 2013).

It was considered unlikely due to the rarity of LSL in children, that several reports from the same study would be identified; however, if it was overtly

declared by referencing the main report, it was agreed that such papers would be included in the review.

A data extraction form (appendix 2.1) was adapted from examples provided in the CRD and the Cochrane Handbook http://handbook.cochrane.org/.

Consensus regarding the data extraction process was reached between the two reviewers without the need for consultation with a third person.

2.5. Assessment of study quality.

A quality assessment checklist was developed by two authors (LM and DT) following the guidelines from the UK CRD REPORT 4 (University of York NHS Centre for Reviews and Dissemination, 2001) and an adaptation of a tool produced by Power and Frank (Power N, 2008) and is found in appendix 2.2: Quality Assessment Checklist.

Papers were assessed for quality and rated as adequate, unclear or inadequate (LM and DT). However, since few papers utilised an assessment scoring system that was validated and comprehensive, papers were recognised as being important literature in the understanding and management of patient with LSL and rated accordingly.

2.6. Analysis

All papers were summarised and the methodological quality compared, based on the inclusion criteria. Studies describing both adults and children were included due to the paucity of publications relating to LSL. Tables, charts and descriptions of assessment tools by which to assess patients with LSL were included as were clinical variables and HRQL measures. The results are displayed in Table 2.2 Summary of Findings.

2.7. Results

A total of 14,833 initial papers were identified along with 4 book sections. Using the duplication tool on EndNote 6, 421 papers were removed leaving 8,412 papers. The titles and abstracts were reviewed and full papers meeting the inclusion criteria obtained and reviewed. A review of the reference lists of key papers and a citation search did not reveal any further studies and a total of 54 papers were included in the final review. The major parameters assessed were bladder and bowel function, neurological function (including muscle power, reflexes, sensory disturbance including pain), orthopaedic deformities, orthotic appliances and HRQL. The search process is shown in Figure 2.2 and was adapted from the PRISMA 2009 flow diagram (Moher et al., 2009). An overview of the 54 papers is shown in table 2.2.

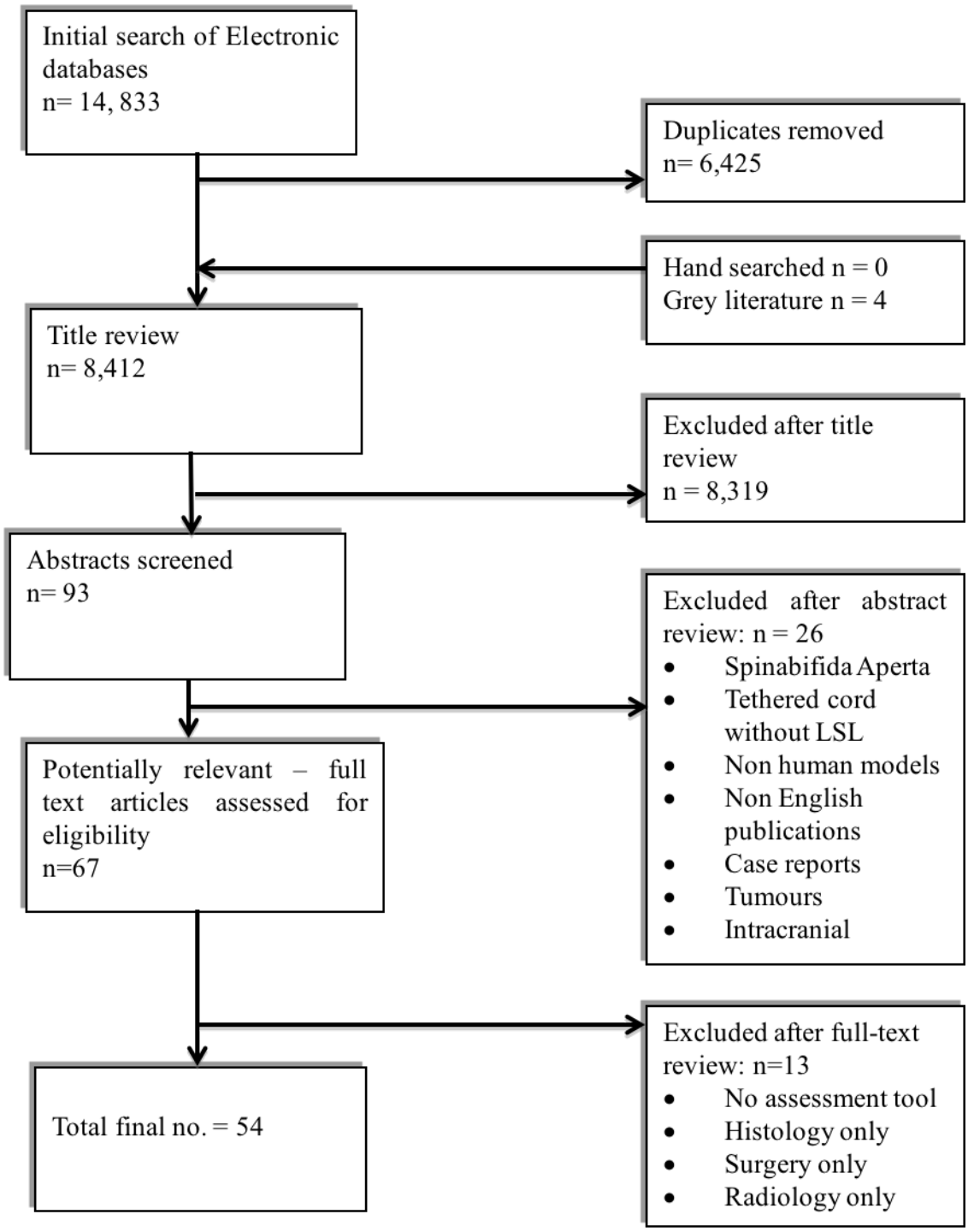


Figure 2.1 Search process

The search study was comprehensive regarding the search terms and types of studies, but a number of abstracts were excluded from the review at this stage, for the following reasons:

Excluded after abstract review: n=26

The exclusions noted in the search strategy Table 2.3 are aligned to the exclusions described in the quality assessment tool (appendix 2.2).

Of the abstracts withdrawn from the review at this stage, 26 were deemed inadequate and withdrawn for the following reasons:

4 were case reviews and as such, provided insufficient sample size to provide any reliable data: **inadequate: not appropriate to study population; inadequate: no assessment tool provided**

14 described spinabifida aperta,-the research group does not include children with myelomeningocele**: inadequate: not appropriate to study population; inadequate: no assessment tool provided; unclear- not all study results provided.**

4 described tethered cord without LSL,-tethered cord can occur in the absence of LSL**: inadequate: not appropriate to study population; inadequate: no assessment tool provided**.

2 Involved animal models: not appropriate to study population; **inadequate: no assessment tool provided; unclear- not all study results provided.**

1 Described intracranial pathology (tumour): inadequate: not appropriate to study population; **inadequate: no assessment tool provided**

1 was not published in English (*English* is generally *considered* to be the gold standard of the scientific community): **inadequate quality**

Excluded after full text review: n=13

Of the abstracts withdrawn from the review at this stage, 13 were deemed inadequate and withdrawn for the following reasons:

6 discussed surgical technique only: **inadequate: not appropriate to study population; inadequate: no assessment tool provided.**

2 discussed histology only: inadequate: **not appropriate to study population; inadequate: no assessment tool provided.**

1 discussed radiology only: inadequate: **not appropriate to study population; inadequate: no assessment tool provided.**

4 did not provide an assessment tool: **inadequate: not appropriate to study population; inadequate: no assessment tool provided.**

54 papers selected for full review.

Table 2.3 Summary of findings

Quality assessment criteria (appendix 2.2) Adequate / unclear / inadequate:

Included * : all criteria rated adequate

Included ** : 1 criteria adequate & 2 unclear, **OR** 1 criteria adequate & 1 unclear & 1 inadequate, **OR** 2 criteria inadequate & 1 unclear, **OR** 2 criteria adequate & 1 inadequate, **OR 3 criteria unclear**

Lead author	Number of patients	Children only	Pure lipoma study	Number of parameters	Orthopaedic abnormalities	Urinary function	Bowel function	Sensory changes	Neurology	Health related QoL	Pain	Scale used	Parameters
(Wang et al., 2013) **	73	Y	Y	4		✓	✓		✓		✓	Uses HRQl scale	Compares children with myelomeningocele to those with lipomyelomeningocele
(Yang et al., 2013) *	73	Y	Y	4		✓	✓		✓		✓	Authors provide their own scale	Table describes improvement, stabilization and deterioration in symptoms
(Kumar et al., 2012) **	93			5	✓	✓	✓	✓	✓			None	Detailed description but no tool provided
(Wykes et al., 2012) **	56			6	✓	✓	✓	✓	✓		✓	None	Detailed description including EMG but no tool provided
(Dushi et al., 2011) **	29	Y	Y	4	✓	✓		✓	✓			None	Detailed description but no tool provided: anal reflex, gait, foot hypertrophy. Detailed urodynamic provided
(Huang et al.,	25	Y	Y	5	✓	✓	✓	✓	✓			Hoffman scale	Refers to cosmesis

Lead author	Number of patients	Children only	Pure lipoma study	Number of parameters	Orthopaedic abnormalities	Urinary function	Bowel function	Sensory changes	Neurology	Health related QoL	Pain	Scale used	Parameters
2010) *													
(Kokubun et al., 2011) **	8		Y	3	✓			✓	✓				Graphs depicting motor, sensory, myelopathy changes
(Pang et al., 2010) *	238	Y		6	✓	✓	✓	✓	✓		✓	Authors provide their own scale	Neurological changes
(Pang et al., 2009) *	238	Y		5	✓	✓		✓	✓		✓	Authors provide their own scale	Neurological changes
(Al-Holou et al., 2009) *	84	Y		6	✓	✓	✓	✓	✓		✓	Modified NEM and Hoffman scales	
(Gourineni et al., 2009) *	159			1	✓							Authors provide their own scale	Complex foot deformities described; muscle weakness
(Maher et al., 2009) *	30			4		✓			✓		✓	Authors provide their own scale	Sphincter EMG
(Muthukumar, 2009b) **	80	Y	Y	5	✓	✓	✓	✓	✓			Hoffman scale	
(Samuels et al., 2009) *	110	Y		4		✓		✓	✓		✓	Reference to modified McCormick	Perineal sensation

Lead author	Number of patients	Children only	Pure lipoma study	Number of parameters	Orthopaedic abnormalities	Urinary function	Bowel function	Sensory changes	Neurology	Health related QoL	Pain	Scale used	Parameters
												scale	
(McGirt et al., 2009) **	27	Y		1	✓								Orthopaedic paper
(Koyanagi et al., 2008) **	34		Y	3		✓			✓			Authors provide their own scale	Neurological deterioration
(Tseng et al., 2008) *	31			6	✓	✓	✓	✓	✓		✓	Authors provide their own scale	
(Daszkiewicz et al., 2007) *	59	Y	Y	6		✓		✓	✓			Loreto scale	Paraestheias, pain, urodynamics, anal sphincter, socio-economic burden
(Rendeli et al., 2007) *	64	Y	Y	5		✓						Authors provide their own scale	Bowel function, pain, motor. Sensory, neurology
(Kasliwal and Mahapatra, 2007) *	63	Y		5				✓	✓			Authors provide their own scale	Neurological symptoms, autonomic symptoms, social life
(Maher et al., 2007) *	22	Y		5		✓	✓	✓	✓		✓	Authors provide their own scale	

Lead author	Number of patients	Children only	Pure lipoma study	Number of parameters	Orthopaedic abnormalities	Urinary function	Bowel function	Sensory changes	Neurology	Health related QoL	Pain	Scale used	Parameters
(Tubbs et al., 2004) **	50	Y		1	✓								Orthopaedic study
(Kang et al., 2006) *	42	Y	Y	1		✓						Authors provide their own urodynamic scales	Urodynamic study
(Morimoto et al., 2005) **	76	Y		3	✓	✓						Authors provide their own scale	Neurological deficits
(Kulkarni et al., 2004a) *	53	Y	Y	8	✓	✓	✓	✓	✓		✓	Modified NEM scale	Gait abnormality, lower limb deformity
(Schoenmakers et al., 2004) **	10	Y		3					✓		✓	Authors provide their own scale	Social function, PEDI score (Pediatric Evaluation of Disability Inventory)
(Babic et al., 2003) **	47	Y		5	✓	✓	✓		✓		✓	Authors provide their own scale	
(Karagiozov, 2004) **	11	Y		3	✓	✓			✓			Authors provide their own scale	
(Dorward et al., 2002) **	50	Y	Y	2	✓	✓						Authors provide their	Neuro-orthopaedic

Lead author	Number of patients	Children only	Pure lipoma study	Number of parameters	Orthopaedic abnormalities	Urinary function	Bowel function	Sensory changes	Neurology	Health related QoL	Pain	Scale used	Parameters
												own scale	
(Kang et al., 2003) *	75		Y	8	✓	✓	✓	✓	✓	✓	✓	Authors provide their own scale	Reflexes, neurological deficits
(Arai et al., 2001) *	120	Y	Y	5	✓	✓	✓	✓	✓			Hoffman scale	
(Bulsara et al., 2001) **	22	Y		5	✓	✓		✓	✓	✓	✓	Authors provide their own scale	Scoliosis
(Cochrane et al., 2000) *	50	Y	Y	3	✓	✓						Authors provide their own scale	Neurology
(Koyanagi et al., 2000) **	34		Y	5	✓	✓			✓			Authors provide their own scale	Muscle atrophy, neurological deficit
(Xenos et al., 2000b) **	59	Y	Y	8	✓	✓	✓	✓	✓		✓	NEM scale	Paralysis, muscle wasting
(Kang et al., 1999) *	60	Y		6		✓		✓	✓			Authors provide their own scale	Urodynamic studies, reflexes, nerve root abnormalities
(Van Calenbergh et	32	N	Y	7	✓	✓	✓	✓	✓		✓	Authors provide their	Reflexes

Lead author	Number of patients	Children only	Pure lipoma study	Number of parameters	Orthopaedic abnormalities	Urinary function	Bowel function	Sensory changes	Neurology	Health related QoL	Pain	Scale used	Parameters
al., 1999) **												own scale	
(Colak et al., 1998) *	120	Y	Y	9	✓	✓		✓	✓			Authors provide their own scale	Reflexes, anal tone, neurological findings, gait disturbance, other
(Wu et al., 1998) **	43	Y	Y	1		✓						Authors provide their own scale	Bladder function only assessed
(Johnston and Borzyskowski, 1998) *	51	Y		5		✓	✓					Authors provide their own scale	Orthopaedic, neurology, self-esteem (associated with bladder and bowel continence)
(Pierre-Kahn et al., 1997) *	291	Y	Y	14	✓	✓	✓	✓	✓		✓	NEM scale	Reflexes, neurological complications, fatigue on walking, orthosis, sexual function, anal tone, EMG limbs and perineum
(Satar et al., 1997) *	28	Y	Y	2		✓		✓				Authors provide their own scale	Neurology as normal or abnormal
(La Marca et al., 1997) *	213	Y	Y	6	✓	✓			✓	✓	✓	Authors provide their own scale	Clinical deterioration
(Byrne et al., 1995) **	100	Y	Y	3	✓	✓			✓			Authors provide their own scale	Table but no assessment tool

Lead author	Number of patients	Children only	Pure lipoma study	Number of parameters	Orthopaedic abnormalities	Urinary function	Bowel function	Sensory changes	Neurology	Health related QoL	Pain	Scale used	Parameters
(Kanev and Bierbrauer, 1995) *	177	Y	Y	2	✓	✓							Neurologically intact or abnormal
(Herman et al., 1993) *	153	Y		6	✓	✓			✓		✓	Authors provide their own scale	Gait, neurological status
(Sathi et al., 1993) **	18	Y	Y	N		✓			✓		✓		
(Atala et al., 1992) **	34		Y	5	✓	✓			✓		✓	Authors provide their own scale	Neurological assessment includes reflexes, motor and sensory changes; EMG
(Colak et al., 1992) *	9	Y		7	✓	✓	✓	✓	✓		✓	Authors provide their own scale	Gait.
(Oi and Matsumotot, 1992) **	178	Y		4	✓	✓	✓	✓	✓			SBNS scale	Reflexes
(Sakamoto et al., 1991) **	75	Y	Y	4	✓	✓			✓			Authors provide their own scale	Neurological status
(Harrison et al., 1990) **	25		Y	12	✓	✓	✓	✓	✓		✓	Authors provide their	Post traumatic deficits (transient paraplegia with dysesthesias),

Lead author	Number of patients	Children only	Pure lipoma study	Number of parameters	Orthopaedic abnormalities	Urinary function	Bowel function	Sensory changes	Neurology	Health related QoL	Pain	Scale used	Parameters
												own scale	abnormal gait, neurologically Reflexes, SSEP
(Kanev et al., 1989) *	80	**Y**	**Y**	6	✓	✓	✓				✓	Authors provide their own scale	Gait, neurological deficit
(Foster et al., 1990) *	31	**Y**	**Y**	3		✓	✓		✓				Urology study, urodynamics

From the review of the 54 papers selected, 29 papers were assigned one star (*) and rated as adequate as per quality checklist criteria; the remaining 25 papers were assigned two stars (**) and included as per quality checklist criteria.

The following clinical parameters were identified in assessing the child's status. The methods of assessment were also identified and are discussed below.

2.7.1. Orthopaedic deformities

Musculo-skeletal deformities associated with LSL are known collectively as the neuro-orthopaedic syndrome and are present in one third to one half of children at initial presentation (Gourineni et al., 2009, Pierre-Kahn et al., 1997). The resulting abnormal muscular-skeletal physiology can result in asymmetric leg length, talipes, foot size club and progressive scoliosis the latter which can sometimes be improved or stabilised following untethering of the spinal cord (Harrison et al., 1990, Kanev et al., 1990, Pierre-Kahn et al., 1997, La Marca et al., 1997). Of 48 patients of mixed ages evaluated by a paediatric orthopaedic surgeon 50% were determined to have orthopaedic anomalies which required some degree of orthopaedic intervention (Tubbs et al., 2006). This ranged from the use of orthotics to surgical procedures, several patients requiring more than one procedure over a wide range of years.

Of the 54 papers reviewed, 38 include orthopaedic assessment in part of their overall assessment criteria. Typically the features are simplified to "scoliosis" and "orthopaedic deformities" and are recorded as normal, improved, stable or worsened (Herman et al., 1993, Kanev et al., 1990, Pierre-Kahn et al., 1997).

Only two studies relate specifically to changes in orthopaedic status: McGirt assessed scoliosis in patients with LSL using Risser grading (to assess the degree of skeletal maturity and scoliosis) and Cobb measurement (which measures the angle of scoliosis / kyphosis), as his assessment criteria (McGirt et al., 2009); the second study by Tubbs et al (Tubbs et al., 2006) who identified that a caudal lipoma was most likely to be associated with orthopaedic abnormalities, with 50% of patients in their study requiring orthopaedic surgery.

Consistent methods of assessing and monitoring orthopaedic abnormalities associated with LSL would be useful in predicting potential outcomes and providing counselling for children and parents regarding the future.

2.7.2. Pain

Pain is described in 26 of the 54 papers reviewed but validated pain assessment tools are not described. There is occasional differentiation in papers between neuropathic pain (Colak et al., 1998) and lower back / or lower limb pain (Pang et al., 2010) but the majority of papers describe pain symptoms as being worse, stable or improved. In the younger child, pain and sensory disturbance in patients with LSL and tethered cord is described as frequently non-specific, with pain being poorly localised and sensory loss often failing to follow a dermatomal distribution (McLone D, 2001). The older child however will present with symptoms more commonly seen in adults with tethered cord, in particular pain on physical exertion. Pain however is often responsive to spinal cord untethering although the incidence of this is not evident from the review. There is however a greater chance of significant morbidity and reduced resolution of pain symptoms in those children who have undergone repeated untethering procedures (Maher et al., 2007).

Pang (Pang et al., 2009, Pang et al., 2010) provides the greatest detail, displaying a table including paraesthesia and dysesthesia, but then reverts to normal, improved, worsened or stable when assessing his patients again at three months post operatively. However, his patient's range from 3 months to 76 years and no assessment measures are suggested for assessment of the young child.

Pain may be associated with several factors in children with LSL, including pain on exertion, pain during adolescence and associated tethered cord, and pain due to orthopaedic abnormalities. These factors are not addressed in the review papers, making analysis difficult without more detail regarding the source, frequency and type of pain and whether pain is restricting the child from participation in physical activities and schooling.

2.7.3. Neurology (including muscular)

Of the 44 papers in the systematic review which assessed neurology, 29 papers also assessed sensation. All 44 papers highlighted changes, deterioration or stabilisation in neurology, with some providing more specific details for gait disturbances and myelopathic changes (Arai et al., 2001, Colak et al., 1998, Harrison et al., 1990, Kanev and Bierbrauer, 1995, Kulkarni et al., 2004a, Pang et al., 2010, Rendeli et al., 2007). Although motor impairment is common and muscle charting an important assessment tool, minimal attention is given to this detail in the literature, with no scales found detailing muscle strength, power and tone in line with existing validated lines. Some papers describe changes in reflexes as a measure of neurological status (Arai et al., 2001, Colak et al., 1998, Kang et al., 2006, Kulkarni et al., 2004a, Sathi et al., 1993, Van Calenbergh et al., 1999).

Several papers acknowledge that loss of neurological function often occurs with age and is likely to be secondary to increased stretch on spinal cord with axial growth spurts (Cochrane, 2008, Kanev and Bierbrauer, 1995). In addition, long tract signs involving the corticospinal tracts vary in children with LSL, often present with a mixed pattern of changes, including motor dysfunction, hyperreflexia and sensory loss of lower limbs. Whilst authors agree with the importance of surveillance of these children, particularly during or following a growth spurt there is a lack of consistency identified in the review regarding specifics of what to assess.

2.7.3.1. Neurology: - cognition

Cognitive abilities associated with spinal lipoma were addressed in one small study (Friedrich et al., 1993). Ten children were studied using the Child Behaviour Checklist, the Developmental Test of Visual –motor Integration and the WISC_R (the Wide Range Achievement Test-Revised). All ten children were within the normal range for academic, intellectual and behavioural assessments, but had low average perceptual motor skills a feature more commonly associated with myelomeningocele, a more severe type of spinal

dysraphism. Whilst the authors recognise the limitations of their study due to small sample size, they surmise that the study group are less competent in undertaking executive functions when compared to healthy children.

2.7.4. Urological function

The majority of papers in the review (50 out of a total of 54) assessed urology and bladder function, which emphasises the importance of regular surveillance of bladder function in the child's overall management, the aim being to identify and reduce the incidence of repeated urinary tract infections, incontinence and progressive renal damage. Urodynamic assessment, including video urodynamics is an established and validated assessment tool and abnormal urodynamic tests may be the only feature suggestive of neurological impairment; Indeed, deterioration in urodynamic function is often the most common symptom that heralds the onset of tethering/ retethering of the spinal cord (Kang et al., 2006, Wu et al., 1998). Urodynamic assessment is fairly standardised in the review with cystometrogram (a graphic measure of bladder pressure at different volumes) and sphincter EMG (electromyography: a measure of the muscles in the bladder wall) being commonly used. Several papers describe urodynamic function in terms such as hyperflexia, areflexia, and normotonia as well as detrusor-sphincter dyssynergia, sphincter abnormalities, frequency of urinary tract infections and bladder dysfunction (Johnston and Borzyskowski, 1998, Kang et al., 2006, Rendeli et al., 2005, Wu et al., 1998). Although validated methods of assessment can provide early evidence of urological impairment and may identify deficits not yet apparent on neurological examination, these are used variably in published literature (Al-Holou et al., 2009, Atala et al., 1992).

2.7.5. Bowel function

A total of 24 papers review details regarding bowel function, with assessments generally incorporated into an overall results table and described as bowel continence or incontinence, or improved, stable or deteriorated. There is minimal description of abnormal bowel function such as constipation or

diarrhoea. Deficits in anal sphincter control are recognised in adults with tethered cord as producing a psychoemotional effect and social ostracism (Daszkiewicz et al., 2007) and yet minimal attention is given in the review to monitoring the severity of incontinence, or discussion on the effect this may have on the child.

2.7.6. Health Related Quality of Life (HRQL)

Despite the fact that LSL is a chronic disease that has the potential to change throughout the child's lifetime only 2 papers in the review include a discussion of HRQL in relation to children with spinal lipoma (Kasliwal and Mahapatra, 2007, Wang et al., 2013). The complex spectrum of children with spinabifida extends from those not dissimilar to the group with LSL (with the potential of reduced mobility, bladder and bowel dysfunction, pain, orthopaedic abnormalities) to those children with myelomeningocele who are likely to be more disabled both physically and cognitively. Each of these factors alone or in combination potentially has far-reaching consequences in terms of relationships, longer-term care and future employment and interdependence. There is a wealth of research involving children with spinabifida including measures of HRQL, and a literature review published by Sawin and Bellin (Sawin and Bellin, 2010) identifies 41 articles with 17 different instruments used to assess HRQL in this group of children. The majority of authors identified that children scored lower on the physical domains than their peers with varying discrepancies in other domains measured, that there was a potential requirement for a score by which to assess the effect of sphincter incontinence on HRQL and that there was no appropriate measurement by which to assess children who were transitioning to adult care. Two questionnaires specific to children with spinabifida were identified in the review: The Health Related Quality of Life-SpinaBifida questionnaire (HRQOL-SB) is a validated instrument developed to include the many sequelae associated with the disease (Parkin et al., 1997). The questionnaire included versions for children aged between 5 to 12, and 13 to 17 years of age, and provides a 5-point Likert scale. The higher the score generated, the higher the HRQL score. Criticism of the model suggests that as a significant number of children with spinabifida have neurocognitive deficits,

allowance is not made for those children whose cognitive age is below the that of their typically developing peers.

The second study by Nanigian and colleagues (Nanigian et al., 2008) produced a Fecal Incontinence and Constipation Quality of Life (FIC QOL) scale. 41 patients and 52 controls completed the questionnaires twice over a 4-6 week period and was found to be a valid and reliability measure for the effects of faecal incontinence on the HRQL of children with spinabifida and their parents. However, only a parent version is available and there is no analysis to determine which of the other domains measured, is supported The value of children's self-assessment of their HRQL is recognized by researchers as providing valuable information as should be utilised in addition to proxy assessment (Eiser and Jenney, 2007).

The severity of symptoms in children with spinabifida is hugely variable including the presence of hydrocephalus and a range of neurocognitive abilities. This renders the use of assessment tools used specifically for children with spinabifida inappropriate for use with our research group, as these factors are not recognised in association with children with LSL.

The two studies identified in the review of LSL incorporating HRQL measures are as follows: In a study of 63 children with spinal lipoma in India, the authors suggests an improvement in HRQL following surgery and describe a "better social life" with improvement in motor and urinary symptoms leading to an improvement in social functioning (Kasliwal and Mahapatra, 2007). The authors do not elaborate with regard to how they measured their findings but suggest similar findings in other papers but these are not referenced. The second study assesses the HRQL of 60 children in Taiwan (mean age, 13.2 years) with spinal dysraphism, using the Child Health Questionnaire-Parent Form 50 (CHQ-PF50) (Wang et al., 2013). The study included 28 children with lipomyelomeningocele and 32 with myelomeningocele and identified that the children with greatest disability (those with myelomeningocele) had reduced physical and psychosocial wellbeing. A limitation of the study is that although children with lipomyelomeningocele were included, those without any neurological deficit

were excluded, thus HRQL in those children with normal neurology but potentially abnormal urology (which is the most common deficit amongst children with lipomyelomeningocele), were not included. Of the 28 children who were included, the authors state that a “considerable number” experienced bladder symptoms, but had a comparable psychosocial summary score compared with a control group of healthy children. The study results did identify a strong association between neurogenic bowel and decreased HRQL, however the study did not differentiate between those with myelomeningocele who are likely to have severe bowel dysfunction, and those with lipomyelomeningocele, who are likely to have minor, if any bowel dysfunction. Lastly, they identified that the presence of limb or spinal abnormalities was associated with a decline in HRQL but again, there was no differentiation between the two study groups. The authors acknowledge limitation of their study in using parents’ proxy reports of HRQL as opposed to children’s self-reports, suggesting that the age of the children and potential cognitive difficulties associated with myelomeningocele, rendered children’s self-report inappropriate for their study. The paper by Wang et al is the sole paper identified in the review, which highlights the importance of measuring the HRQL of children with spinal dysraphism including spinal lipoma and associated deficits (Wang et al., 2013).

The core assessment methods identified in the review which measure different sequelae associated with LSL in children are discussed below:

2.8. Core assessment methods identified in the review

The majority of authors assess clinical symptoms in terms of non-progressive (stable), progressive (deteriorating) and improving. Parameters are displayed in their own tables, including assessment of neurology (motor and sensory status, pain), orthopaedic status, urinary function and bowel function. The number of total parameters measured varies between papers although some papers include adults in their review and consequently include sexual function in their data. Some studies have correlated their assessment outcomes into a score and the most comprehensive of these is the Personal NEM Score.

The NEM Score was developed by Pierre -Kahn's team (Pierre-Kahn et al., 1997) and evaluates functional repercussions of children with spinal lipomas (appendix 2.3) and which Xenos et al (Xenos et al., 2000b) suggest provides rapid comparison between patients. The NEM score is modified for infants and babies where assessment of neurology and continence is more difficult. The scores address major handicaps including motor, sensory, vesical and anal, with the lower score depicting the greater handicap. Normal function is depicted as 5 for both motor and urinary functions, 4 for sensory function and 3 for anal function, with an overall potential score of 17. The authors acknowledge the simplicity of the scoring system but suggest it is an improvement on previous studies, which evaluate neurological level of deficits but not their functional repercussions (Oi and Matsumotot, 1992). It is unclear from the literature whether the NEM assessment scoring system has been validated, assessed for inter-reliability, or for ease and speed of use.

Adaptations to the NEM score include the addition of gait abnormality, paralysis and muscle wasting, (Al-Holou et al., 2009, Kulkarni et al., 2004a, Xenos et al., 2000b).

Oi (Oi and Matsumotot, 1992) utilises the SpinaBifida Neurological Scale-(SBNS), which evaluates all the elements required for assessment of children with spinal dysraphism including motor function, reflexes, bladder and bowel function, but is more suited to children with myelomeningocele who are more severely affected than those with LSL. The child's clinical status is divided into 5 grades, ranging from grade 1-3 as ambulant and grade 4- 5 as non-ambulant. Fecal and urinary incontinence is also analysed as part of the total outcome score. The score has no reference to pain.

Of the clinical assessments published, those by Pang and Pierre Kahn attempt to assess the majority of sequelae associated with LSL (Pang et al., 2009, Pang et al., 2010, Pierre-Kahn et al., 1997). However only Pierre-Kahn provides a scale encompassing all age groups and neither provides tools by which to assess pain. The author could find no reference to validation of the scores, assessment of inter-relater reliability, or assessment of ease and speed of use.

2.9. Summary

LSL is a rare and complex condition and the outcome for the child is dependent on many factors, including individual pathophysiology, age of the child, natural history of the disease and expertise of the surgeon and clinical team (May et al., 2013).

Finn (Finn M, 2007) amongst other authors recognises the three main neurological symptoms associated with spinal lipoma including LSL as being urological, orthopaedic and neurological. Despite the fact that clinical deterioration is often subtle, most authors do not suggest or utilise specific tools by which to monitor related symptoms. There is however a general consensus on the need for close monitoring by a multidisciplinary team of orthopaedic surgeons, neurosurgeons, urologists and physical therapists.

The diagnosis of LSL remains primarily a clinical one, requiring evaluation throughout childhood and beyond (May et al., 2013). Although clinical assessment remains the optimum method of assessing and monitoring children with LSL, the lack of a standardisation of parameters and a validated tool by which to measure them leaves the clinician without a robust method by which to monitor his / her patients, and does not offer him a comparison of his management protocol including post operatively results, with those from other centres. The literature reflects the specialty of the reporting authors (neurosurgery, urology, orthopaedics) rather than the overall clinical condition of the child; furthermore, the clinical heterogeneity (both methodologically in terms of study design and risk of bias, and clinically in terms of variability between patients, surgical intervention and outcomes) is highlighted in the review. LSL can be associated with morbidity and care is aimed at maximising health and minimizing disability over the spectrum of the child's life. HRQL measures can be used to identify if the child is affected by the impact of the disease, in addition to the effectiveness of treatment strategies and interventions. The following tables show the drivers (Table 2.3) and the barriers (Table 2.4) for measuring HRQL in chronic disease.

Table 2.4 Factors depicting drivers for measuring HRQL

Driving Factor	Potential Result
Evaluation of patient status	Improved communication identified between clinician and parent (Neumann et al., 2012) Improved understanding of unique perspective of parent /child may result in improved outcomes for child (Marino et al., 2009) Empowered parents through mutual understanding and involvement in care e.g.: PROMS (Patient reported Outcome Measures), which provide a measure of HRQL by focusing on the patients perspective with regard to the effectiveness of interventions, in addition to patient involvement and empowerment (Department of Health and 2008).
Assessment tool for parent / child	Time restraints involving the clinician, child and family.
Perception of the importance and validity of qualitative research	Evaluate changes in morbidity Assess efficacy of medical interventions Evaluation of progress Comparison of treatment across individuals and institutions

Table 2.5 Factors depicting barriers for measuring HRQL

Driving Factor	Potential Result
Time constraints	Time constraints involve the clinician, child and family.

Perception of the importance and validity of qualitative research	Some clinicians believe assessing the patient's HRQl is irrelevant, unimportant and does not affect treatment, management or outcomes (Espallargues et al., 2000, Fihn et al., 2004)
Response shift	HRQL self-assessment may alter in line with changing health and the "response shift" highlights the importance of considering when the assessment was undertaken (Eiser and Jenney, 2007). If the measure is repeated, financial and time implications may present an additional difficulty.
Limitations to measuring HRQL	Specific intervention and treatment planning may be restricted where HRQL measure fail to capture specific, individualised problems (Stabler B, 1998)
Economic impact of measuring HRQL	Family income can have a detrimental effect on the child's HRQL, but confounding factors include psychosocial factors (Cassedy et al., 2013) Direct financial cost related to health care and indirect costs due to loss of parental earnings due to care requirements Potential change in public health policy and education to address these point (Witt and DeLeire, 2009)
Financial impact	Assessing HRQL has financial

	implications in terms of administrative costs and time.

Chronic disease in childhood may affect all family members, particularly the parents who play a significant role in the relationship and co-operation between the child and health care professionals (Lollar and Simeonsson, 2005). Therefore, when assessing the HRQL of children with LSL it would seem prudent to also assess the impact of the disease on the child' parents (and this has not been identified in the review) recognising that the attitude of children to illness can be dependent on the behaviour, attitude and well-being of their parents.

2.10. Strengths of the review

This review systematically identified all research conducted since 1990 that focused on the symptomology of children with LSL and the methods / tools by which to assess them. The review identified the importance of such assessments and the lack of consistency by which such assessments are undertaken, including a lack of HRQL measures. There is no reference linking the effects of the clinical deficits experienced by these children, to HRQL outcomes, thus highlighting the need for future research in this area.

2.11. Limitations of the review

The review highlights the paucity of publications regarding children with LSL and the resulting difficulty of comparing findings with an appropriate control group. As the majority of publications are from single institutions and single surgeons, patient selection bias must be considered, as specialist institutions publish articles that are not representative of the wider community and this selective policy, renders their data open to criticism of bias in their selection of published data. Without consistent, unbiased reporting of outcomes, the recognition of asymptomatic children verses those who may require repeated intervention and disability, goes unrecognised.

Several of the studies have a small number of patients, with one paper describing only 9 children in the study (Colak et al., 1992). Such small sample sizes allow only a cautious interpretation of the study and assessment tools used. The reason for including studies with such small sizes was to capture all potentially relevant studies regarding such a rare anomaly.

2.12. Implications for practice

With such a rare anomaly, it would seem prudent to utilise standardised and validated assessment methods by which to audit, research and improve patient care and outcomes. As with any child living with a chronic condition, psychosocial effects should be included when assessing children with LSL, including HRQL, to ensure the provision of holistic care and appropriate resources and support.

All assessment methods need to be easy to use, useful, reproducible, validated and comparable. In addition to standardising assessment and practice, multicentre research would result in a greater understanding and improved management of this rare anomaly. The deficits of children with LSL can be wide ranging and further research is required to identify the effect this has on the daily life of the child.

The review of children with spinabifida undertaken by Sawin and Bellin (Sawin and Bellin, 2010) highlights the importance of assessing the HRQL of children with spinabifida in providing the necessary physical and psychological interventions and strategies, and appropriate resources. The literature review of LSL has identified a gap in the assessment of HRQL in relation to the physical sequelae in this group of children and provided a focus for current research.

As a consequence of the systematic review a clinical assessment sheet was devised and HRQL measures selected, both of which were used in the clinic setting in the research centre.

2.13. Summary

This chapter provides a systematic review of the literature from 1990 to 2013 (and an update in 2015) on assessment tools by which to assess children with LSL, explaining the importance in identifying the key symptoms associated with the condition. A total of 54 papers were included in the final review and the quality assessment and data extraction processes were described. Results of the review were detailed in table 2.2 and described within the clinical parameters and HRQL measures relevant to LSL.

The chapter closes with recognition of the lack of consistent assessment tools and the importance of providing standardised tools in order to identify and consequently treat early signs of deterioration. Despite the negative impact that a chronic disease such as spinabifida has on a child' s HRQL (Sawin and Bellin, 2010), the HRQL of children with LSL is not addressed in the review and leaves the opportunity for future research.

The following chapter will present the aims and methodology of the thesis.

Chapter 3. Methodology

3.1. Introduction

The previous chapters have discussed the pathoembryology and pathophysiology of lumbosacral lipoma (LSL), the physical sequelae associated with the condition, a review of the methods by which to assess these sequalae and a recognition of the lack of HRQL measures by which to assess this group of children. The results of the systematic review indicated that there is a lack of consistency in the evaluation of symptoms in children with LSL and that some areas, for example pain and HRQL are not routinely measured. The study aim is to seek to address these shortcomings by firstly systematically and comprehensively evaluating the physical symptoms of a group of children with LSL and to examine the association between these symptoms and the type of LSL. To understand the effect that this chronic disease may have on the child, health related quality of life (HRQL) including self-esteem will also be evaluated. The findings can then be distilled into an assessment tool to be used in the clinical outpatient setting.

The aim of the current chapter is to summarise the research question and provide the methodology used to address these aims.

3.2. Research questions and aims

The lack of homogeneity amongst children with LSL makes the provision of individualised prognosis difficult, with disparity particularly in motor and urological function resulting in uncertainty for patients and parents regarding long-term morbidity. Most studies relating to LSL in children focus on the pathology of the disease and surgical intervention, and little is known of the effect the disease may have on the child and family. The research questions were described in the introduction section.

3.3. Sample and setting

The study was undertaken in a paediatric tertiary referral hospital in London.

LSL is a rare condition and there are only approximately 150 patients under follow-up at this centre. The power calculation for the study is based on use of The Pediatric Quality Of Life Inventory (PedsQL) as this measure has been validated for use in many different countries and for many different health conditions (Varni et al., 2005). It was therefore selected for use with all of our patients. With 90 patients seen over an eighteen month period and allowing for refusal rates, approximately 60 patients were recruited into our study. This provided a sufficient sample to pick up a correlation (between the PedsQL total score and a clinical symptom score) of 0.4 or greater as being statistically significant, with a power of 90% and a p value of 0.05. In addition, comparisons were made between our population of children with LSL and those of published UK normative values with children of similar age group using the PedsQL, for whom the mean is 82 and the standard deviation (SD) is 13. Assuming a higher S.D. of 20 for the children with LSL, 52 patients were required, to enable us detect a reduction of 9 units on the PedsQL scale (i.e. an effect size of 0.46).

3.3.1. Inclusion Criteria

- All children with LSL between the age of 5 and 18 years old under the care of the tertiary hospital, and their parents. A translator was arranged in advance for those who did not speak or understand the English language.
- Parents(s) or legal representative must be able to provide consent.
- Participant must be able to give assent.

3.3.2. Exclusion Criteria

- Participants were excluded if they / their family were unable or unwilling to give informed consent.

- Children with both LSL and cloacal atresia were excluded due to the medical complexity of the latter, involving extensive ambiguous sexuality, and abdominal abnormalities.

3.4. Recruitment

Children eligible to participate were identified from searching future clinic appointments, clinical databases and medical notes prior to neurosurgical outpatient clinics (by a member of the clinical team). All children over the age of 5 years and under the age of 18 years and their families were invited to participate over a period of 18 months, regardless of their clinical presentation. In a retrospective study of 53 asymptomatic children with LSL, Kulkarni et al identified a similar incidence of clinical deterioration between children with LSL who had undergone surgical resection of the LSL and those who had not (Kulkarni et al., 2004a). As a consequence, the children in our research group were not selected or identified between those who were symptomatic and had undergone surgery and those who were asymptomatic had not undergone surgery.

Information sheets outlining the study were sent to the parents and age appropriate information sheets sent to the children, a month prior to their routine neurosurgical out-patient clinic. The parents were asked to reply by telephone, e-mail or by using the pre reply envelope supplied; if the family had not replied within 2 weeks, they were resent information. Details of parents who identified they did not wish to participate were noted. The researcher, to determine whether there were factors that influenced this decision, asked this group of parents if they would be willing to give a reason for lack of participation, to enable further understanding of what might be important to them. All explained that as their child was currently asymptomatic, they did not wish to introduce the idea to their child, that he / she might deteriorate clinically over time.

The age of the child whose parents did not wish to participate was noted. This was to ensure that any child of the age of 16 years and above and thus able to consent themselves, could have the opportunity to participate if they so chose.

The age of the children whose parents did not wish to participate were all younger than 16 years of age.

The researcher obtained consent when the participants attended the hospital for their outpatient appointment, prior to commencing any of the research and after the child and family had had the opportunity to ask further questions regarding the research. Written parental consent was required for the child to participate and assent from any child less than 16 years of age. Parents were required to give written consent for their own participation.

3.5. Ethical considerations

The five most important considerations when undertaking research in children are set out by The UK Medical Research Council's Ethics Guide: Medical Research Involving Children (MRC Ethics Guide, 2004) and are as follows:

3.5.1. Risk assessment

"The foreseeable risks should be kept as low as possible and the potential benefits from the development of treatments and furthering of knowledge must outweigh any foreseeable risks" (MRC Ethics Guide, 2004).

It was made clear to the participants in the study that their care would not be affected if they chose not to participate in the research.

If participants became distressed in talking about their (or their child's) condition, they were offered the opportunity to terminate the session. They were offered input from the hospital care team for further support if desired. If completion of the measures indicated the need for further medical, pharmacological or other therapeutic intervention, appropriate referrals were made.

It is recognised that any research can be considered burdensome for children and their families; however, the risks to participants were considered minimal. The timing of assessments was estimated to allow the participants to be aware of time constraints they may face, before consenting to participate in the study.

3.5.2. Consent

"The voluntary agreement of an adult or competent child, based on adequate knowledge and understanding of relevant information, to participate in the research"(MRC Ethics Guide, 2004).

Verbal and written information regarding the study was given to all parents following which they had the opportunity to ask any questions. Signed consent was then obtained for their own participation in the study. Following the provision of verbal and written age appropriate information and an opportunity to ask any questions, a signed assent form was obtained from all children under 16 years of age and a signed consent from those over 16 years of age. A parental, signed consent form was obtained from the parents of children under 16 years of age.

Children and parents were reassured that participation / lack of participation / withdrawal from the study would not influence their clinical care and support.

3.5.3. Confidentiality

"Medical professionals have a duty of confidentiality to all patients including children. If competent children do not wish to involve their parents/guardians this should be respected"(MRC Ethics Guide, 2004).

Confidentiality was set as a ground rule and the participants informed that information gathered from the research was pseudo-anonymised by allocation of a study number to prevent identification of the participant.

Raw data were kept in a locked filing cabinet and disposed of as confidential waste once the study ended. Electronic data were stored on a password protected Trust computer, accessible only to the researcher.

Should the child reveal information that might put him/her at risk, the researcher encouraged him/her to share this with his/her parents and / or members of the health care team. All researchers working with children have a responsibility to

liaise urgently with social services should they suspect the child is suffering / likely to suffer harm.

3.5.4. Safety

“Children's safety in relation to researchers: Any individual working directly with children will undergo security screening, including criminal records review”(MRC Ethics Guide, 2004).

In addition to the above, information sheets for the child and family included details of who to direct complaints to.

3.5.5. Ethics committee review

“According to guidance from The Council of Europe Protocol to the Convention on Human Rights and Biomedicine on Biomedical Research, every research project must be submitted for independent examination of its scientific merit, including assessment of the importance of the aim of research and ethical acceptability to an ethics committee”(MRC Ethics Guide, 2004).

The study protocol was reviewed and approved by the Research and Development (R&D) team of the participating site on 8/01/2013 (appendix 3.1)

The study protocol was reviewed and approved by The National Research Ethics Service, London 03/04/2013 (appendix 3.2)

NRES was established to protect the research participant’s rights, well-being, safety and dignity and to promote ethical and beneficial research. Parent and age-appropriate child study information sheets were reviewed and approved by the committee.

Child and parent information sheets are provided in appendix 3.3 and questionnaires are provided in appendix 3.4

3.6. Data collection

Information was obtained from the medical notes regarding any co-morbidity, any medications used, previous surgery or interventions, the presence of absence of a syrinx and the type of lipoma. Demographic information was also obtained from the medical notes.

3.6.1. Assessment of clinical symptoms

All children with LSL at the hospital undergo regular, standardised urodynamics and neurological assessments and where required, orthopaedic surveillance / intervention. Data from these investigations was collected from the medical notes and / or database to form part of the thesis. The NEM scale produced by Necker hospital in Paris and published in 2007 (Pierre-Kahn et al., 1997) was used to provide further clinical data for all children (table 3.1). The scale has not been validated or assessed for inter-reliability; however, it has been previously piloted by the research team involved in the current study and found to provide useful information regarding both clinical deficits and functional repercussions, and was found to provide rapid assessment in a clinical environment.

Table 3.1 The NEM scale

Rating	Motor	Sensory	Bladder	Bowel
1	Wheelchair. *Major deficit*•	Skin ulceration or amputation	Day and night incontinence. Bladder augmentation/ mitrofanoff Incontinence•	**Incontinence / ostomy**
2	Major orthosis	Pain	Nocturnal incontinence. Retention•	**Painful constipation**
3	Distal orthosis. Club foot, atrophy, distal deficit•	Painless sensory deficit	CIC	**Normal**
4	Fatigue on walking	Normal	Dysuria, infections, stress incontinence	
5	Normal		Normal	

Clinical data for all children recruited into the study were also entered into table 3.2, which is an adaptation from the table suggested for data collection in the systematic review of children with lipomyelomeningocele (May et al., 2013). Adaptations involved the addition of assessment of lower limb reflexes; in addition, all clinical assessments were rated numerically with 1 depicting no abnormality, 2 depicting abnormal but fixed deformity, and 3 depicting a deterioration in function. Patients were considered to have deteriorated if they developed evidence of motor or gait deterioration, increased pain in leg/back (particularly on activity) urological deterioration as confirmed by urological

assessment and to a lesser extent, evidence of bowel disturbance (constipation / incontinence).

Data for all questionnaires is discussed separately and documented accordingly.

Table 3.2 Clinician's observation and data collection

Clinician's Observations		Normal=1 Abnormal but stable=2 Deteriorated =3
Muscle strength MRC grading (MRC, 1981)	MRC grading	**1/2/3/**
Foot deformity **Ankle deformity** **Scoliosis**	Yes /no Yes /no Yes /no	**1/2/3/** **1/2/3/** **1/2/3/**
Patellar reflex **Achilles reflex**	Yes / no Yes /no	**1/2/3** **1/2/3**
Pain leg / back/ both (identify which) **Worse with exercise**	Yes /no Yes/ no	**1/2/3** **1/2/3**
Urinary symptoms	Yes/ no	**1/2/3**
Bowel symptoms **Constipation** **Incontinence**	Yes / no Yes / no Yes / no	**1/2/3** **1/2/3** **1/2/3**

3.6.1.1. Neurology

Neurology must be assessed accounting for the developmental milestones of childhood and include assessment of reflexes and muscle strength (MRC, 1981). Assessment of neurological status and sensory changes including pain were rated and documented as described above.

The following two assessments were undertaken to identify if they provided specific information regarding the physical activity of children with LSL.

3.6.1.1.1. GAITRite walkway

Movement is multifactorial and includes measurements of muscle activity, gait, kinematics (joint range changes) and kinetics (forces and movements across joints). The GAITRite walkway (electronic pressure sensitive walkway) produces 20 gait parameters using 8 sensory pads and is a validated method of assessing gait and generates measurements of time, speed, and length of stepping, to detect gross and subtle compensatory changes in gait pattern. The continuous data generated can be interpreted against established graphs for healthy children.

Several studies have identified the GAITRite as a useful method by which to identify functional deficits in walking, components of gait, balance and mobility: Ferrarin et al (Ferrarin et al., 2013) used the GAITRite walkway to compare changes in gait in a group of 19 children with demyelinating peripheral neuropathy following surgery and were able to identify subtle changes using the GAITRite walkway; in a UK study of children with haemophilia, the GAITRite walkway was used to identify children with joint arthopathy and the results matched against a control group (Bladen et al., 2007). Whilst this is a small study, the use of the GAITRite was important in identifying subtle changes that that could assist early intervention and management of joint arthopathy in this group of children; a further study in the UK using the GAITRite to assess changes in gait pattern of children with mucopolysaccharide disease, found that the system provided qualitative and quantitative data, was practical to use in the clinical setting but the authors acknowledged that larger studies were required (Wood et al., 2009b). The GAITRite was also identified as an effective method of measuring gait speed and gait maturation of healthy children: Lythgo et al (Lythgo et al., 2011) studied 656 healthy children and adults in a non-clinical environment and provided normative values which can be used as a control group when assessing children and adults with mobility deficits.

The GAITRite was therefore identified as a potential tool for the research group.

3.6.1.1.2. Pain

The child's medical history included taking a pain history, and involved ascertaining the quality, frequency and intensity of pain, aggravating / relieving features, localisation of pain / sensory changes and the response to management strategies. All children recruited to the study were asked about the presence of pain and scored by the researcher accordingly: a binary score was documented (whether they had pain or not); in addition, the children were asked if they had pain, if they had pain but the level of pain was stable, or if their pain was increasing (i.e. deteriorating). Their ratings were scored accordingly.

In addition, all children and their parents as proxy respondents completed an age appropriate pain questionnaire, the PedsQL pain questionnaire (PPQ)(Varni et al., 1987). The application of this questionnaire is outlined below in section entitled child/ parent-proxy questionnaires.

3.6.1.2. Orthopaedic

All 54 children were examined for the presence of any form of foot deformity, ankle deformity and scoliosis. Absence of a deficit was recorded numerically by the number 1, a stable abnormality as 2, and deterioration as 3.

3.6.1.3. Urological function

History taking includes the presence or absence of dry periods between nappy changes, an intermittent urinary stream or dribbling of urine, and a history of urinary tract infections. In the child who has attained urinary continence, history taking includes details regarding the frequency of micturition, urgency of micturition, any periods of nocturnal enuresis, stress incontinence and urinary tract infections.

Renal ultrasound is undertaken as a baseline on which to assess any future renal damage or reflux, and bladder ultrasound to provide data on bladder

capacity and post void residue volumes. Bladder capacity is calculated on the following formula: (Age in years +2) x 30 = mls (Martinez-Garcia et al., 2013). In addition, bladder wall thickness will be measured, with a bladder wall thickness of more than 3-4 millimetres, measured at 50% of expected bladder capacity, indicating detrusor over activity, bladder outflow over activity, or obstruction (Kirchin et al., 2001).

For the non-toilet trained child, 2-4 hour observations using enuresis alarms such as a “wet nappy alarm” (Malem Medical, Ltd., Nottingham, UK) are undertaken and provide a measurement of voiding frequency; with nappy weight recordings and post void ultrasounds are also undertaken.

For the toilet trained child uroflometry is undertaken, with 3 successive recordings and post void ultrasounds. The flow rate of urine is measured electronically, along with the flow time, flow volume, time to maximum flow and the intermittent flow and the results compared to normative data. A graph recording is generated from the data and the results interpreted along with the patient’s age, weight and sex. Detrusor over activity (hyperreflexia) and under activity is recorded to complete an overall picture of the urinary status of the child.

Each of the children recruited into the research study underwent an age appropriate standardised urodynamic assessment and the results documented by the urology team as part of their routine follow-up. The child’s overall status of bladder function was recorded numerically by the researcher with number 1 indicating no abnormality, 2 indicating a stable abnormality, and 3 deterioration in urological function.

Should invasive bladder function assessments be required to provide more detail, a bladder and rectal catheter would be used to produce data regarding intravesical and abdominal pressures.

3.6.1.4. Bowel function

History taking is essential in establishing bowel function and the NICE guidelines provide information regarding the diagnosis and management of faecal continence and incontinence (NICE, 2014). To establish a diagnosis of gastrointestinal disorders including constipation, two or more of the following criteria must occur once per week for at least two months, in a child with a developmental age of at least 4 years (Rasquin et al., 2006).

- Two or fewer defecations per week
- At least one episode of faecal incontinence a week
- History of retentive posturing or excessive volitional stool retention
- History of painful or hard bowel movement
- Presence of faecal mass in the abdomen
- History of large diameter stools that may obstruct the toilet

Under the age of 4 years, bleeding associated with a hard stool, distress on soiling and straining are associated with constipation (NICE, 2010).

The child's overall status of bowel function in the study group was documented by the urodynamic department during their routine standardised assessment, and recorded numerically by the researcher with number 1 depicting no abnormality, 2 a stable abnormality and 3 as deteriorating bowel function.

3.7. HRQL measures

3.7.1. How to measure HRQL

Quantitative methods of measuring HRQL provide a numeric measurement and can be used on their own or in combination with qualitative methodology, which provides a narrative of the participant's experience. Wallander and Koot (Wallander and Koot, 2016) suggest that HRQL is composed of objective (the degree of health or ability) and subjective (psychological and physiological)

elements, and instruments to measure HRQL can include both these important concepts.

3.7.2. Quantitative methods of HRQL measurement

The most frequently used method of quantitative assessment is a questionnaire, which normally uses a Likert-type scale and provides the participant with statements or questions which they select according to their self-perception. The numerical value assigned to their selection thus provides quantitative value to qualitative data, enabling statistical analysis of the data.

3.7.2.1. Single / multidimensional constructs

Measures of HRQL can be single construct (asking a single question which provides a broad perception of the participant's HRQL) or multidimensional (which measures multiple aspects of the participant's HRQL). The latter is more commonly used as it provides greater information and is more useful in research. Multiple independent scales (the Battery method) can also be used and provide large amounts of data, but can be burdensome to young children and in addition, interpretation of results between instruments can be difficult (Ferrans et al., 2005). The researcher must decide which measurement method answers the research question, without being too onerous for the participant (particularly children) to complete.

3.7.2.2. Generic / Disease specific questionnaires

Validated, multidimensional disease and generic specific questionnaires have been developed for children and assess multiple areas of HRQL including physical and psychological aspects. Disease specific measures provide data that is clinically relevant to a specific disease and thus have the advantage of detecting subtle changes that are not captured in generic measures (Palermo et al., 2008). Generic measures provide a broad measure of HRQL irrespective of the underlying disorder and allow for comparison across demographic or clinical populations. Generic measures may lack sensitivity and precision in detecting

longitudinal changes or detect subtle aspects of specific conditions (Ronen and Rosenbaum, 2013).

3.7.2.3. Self / parent-proxy completed questionnaires

HRQL is a subjective concept and ideally should be assessed from the patient's viewpoint with the use of age appropriate questionnaires. Parent-proxy reporting is widespread in paediatrics, as children have previously been considered unreliable respondents due to age, language, health status and cognition (Taylor et al., 2005). However a systematic review of HRQL assessments for children under 12 years of age, identified that even very young children with a variety of physical and cognitive deficits were reliable reporters of their HRQL (Jardine et al., 2014). Parent-proxy report should therefore be used to supplement, not replace the child's self-report. The child's understanding of his / her own health and the effect this has on his / her life may differ from that of his/ her parents, with the child sometimes being more optimistic about the future than their parent (Eiser and Jenney, 2007). Although there may be a degree of disagreement between child and parent-proxy, both child and parent-proxy reports provide separate and important information and ideally should be used together (Gabbe et al., 2010).

3.7.3. Questionnaires completed by the child

The questionnaires / additional assessments undertaken by the children and families who consented to participate in the research is displayed in appendices 3.4.

Questionnaires for studying HRQL in children with LSL were selected which are multidimensional, include a self-report with a parent /proxy version where appropriate, have established validity and reliability, and have published normative values. Developmental changes in language development are important in assessing children and the need for each specific measure when studying children young children and adolescents has resulted in age appropriate questionnaires such as the PedsQL (Varni et al., 2005).

Questionnaires facilitate comparison across groups of children and their parents. Table 3.3 provides a summary of the questionnaires provided to and completed by the recruited children and table 3.4 provide the parent / proxy questionnaires provided.

Table 3.3 Questionnaires completed by the child

Participant	Questionnaire	Time Taken to Complete *(In Minutes)*	Parameters Measured
5-6 years	PedsQL PedsQL pain	5 2-3	Quality of life Pain and sensory scoring
7 years	PedsQL PH2 PedsQl pain	5 10 2-3	Quality of life Self-concept Pain and sensory scoring
8-9 years	PedsQL PH2 PAQ PedsQL pain	5 10 5 2-3	Quality of life Self-concept Physical activity Pain and sensory scoring
10-18 years	PedsQL PH2 CHQ-CF87 PAQ PedsQL pain	5 10 20 5 2-3	Quality of life Self-concept Quality of life Physical activity Pain and sensory scoring

The optimum standard for assessing HRQL is to use both generic and disease specific measures as this provides the richest data (Eiser and Jenney, 2007) but as there are no disease specific measures available for children with LSL, generic questionnaires were used to measure HRQL. Generic questionnaires

were also used for activity and pain scoring as none were available that were specific to children with LSL.

3.7.3.1. PedsQL 4.0. Generic core scale

The Pediatric Quality Of Life Inventory (PedsQL) was originally developed in the Unites States of America as a model by which to integrate generic and disease specific modules of assessment. The generic PedsQL 4.0 scale is applicable for use in acute and chronic paediatric diseases as well as for healthy children, has age appropriate versions for children aged between 2-4 years old, 5-7 years old, 8-12 years old and 13-18 years old and parallel forms for the parent of these age groups. It has been translated into over 60 languages with published data for over 25,000 children and adolescents (Varni et al., 2005).

The PedsQL scale measures the severity of a problem for each item over the past month and answers range from 0 (never a problem) to 4 (almost always a problem). 23 items contribute to the 4 sub scores (physical, emotional, social, school) from which a total score can be calculated. Each of the individual item scores are converted to a 0 to 100 score and mean scores are calculated for the summary score. A lower score represents a lower quality of life.

A UK-English version of PedsQL was produced in 2005 and found to be of equal reliability and validity with psychometric properties similar to those reported for the original PedsQL (Upton et al., 2005). A literature review of 19 HRQL measures undertaken by Upton and colleagues (Upton et al., 2008) highlighted the importance of using consistent measures when assessing the HRQL of children and their families and identified the PedsQL as the most commonly used measure to provide consistency and parent-child agreement.

The PedsQL child and parent / proxy questionnaire was identified as a suitable tool by which to evaluate the HRQL of 104 children with spinabifida and their parents (Freeman et al., 2013). However, the authors suggest additional domains in this complex group of children should be included to measure, pain and family environment and influence.

The PedsQL was used under license (The PedsQL Measurement Model for the Pediatric Quality of Life Inventory).

3.7.3.2. Child Health Questionnaire: CHQ-CF87

The CHQ-CF87 is a validated generic measurement of HRQL for children and adolescents and is based on the World Health Organisation definition of health.

The questionnaire includes measures of physical, psychological and social well-being, is validated with good reliability and has normative data (Waters et al., 2003, Landgraf and Abetz 1997).

The self-report version (CHQ-CF87) contains 87 items and is valid for children and adolescents aged 10-18 years and has domains including general health perceptions, bodily pain, physical function, role/social-physical, role/social-emotional, role/social behavioural, mental health, self-esteem, general behaviour and family activity and family cohesion. The questions are based on a 4 week recall and the participant scores his / her response using a 4-6 point Likert scale indicating agreement to the questions from “very often” (score 0) to “not at all” (score 4). Scores within each subscale are summed and the raw scores converted to a score of between 0 and 100, with the lower scale depicting a lower functional and well-being score.

Limitations to the CHQ-CF87 questionnaire were identified in a study of 49 children with bladder extrophy, due to the lack of disease specificity and sensitivity at the time of publication (Schaeffer 2012). Despite these limitations, the questionnaire was identified as useful in highlighting the child’s personal and academic achievements, interpersonal relationships, and emotional and behavioural elements in addition to physical deficits. A further limitation to using the CHQ questionnaires was highlighted in a study of 613 children with epilepsy in which the CHQ-CF87 was scored by the children and a parental score undertaken by the parents using the CHQ-CF50 (Baca et al., 2010). The authors identified that parents rated their child’s HRQL lower than the child

rated himself / herself and there were limitations in comparing the two scales as they contain similar, but not identical items.

A large study of 2,361 adolescent participants completing the CHQ-CF87 was undertaken to provide normative data and to assess the impact of common health anxieties (Waters et al., 2001). The results identified that HRQL deteriorated in line with a decline in physical health and that an increase in HRQL correlated with improved physical health, with the CHQ-CF87 providing a reliable measure by which to measure HRQL.

The current study used the CHQ-CF87 as it contains multiple items as identified above and has comparative data available for both healthy children and those with a chronic illness (Bannink et al., 2010, Waters et al., 2001). Although the CHQ-CF87 has not been validated for use in the UK, normative ranges from other English speaking populations are established in all domains of the CHQ-CF87 (Schaeffer et al., 2012).

The CHQ was used under license (Healthactchq. Inc.)

3.7.3.3. Pediatric Pain Questionnaire (PPQ)

Assessing pain in young children can be a challenge and this has resulted in the creation of several age-specific pain management tools and scores. The Pediatric Pain Questionnaire (PPQ) was developed by Varni and Thompson (Varni et al., 1987)(also known as the PPQ-VAS scale) and provides a validated, comprehensive, multidimensional instrument for measuring the child and parent's perception of the intensity and location of the child's pain. The chart was first developed to measure the muscular skeletal pain of children with rheumatoid arthritis and was found to provide a comprehensive method of pain assessment (Varni et al., 1987).

The self-rated Visual Analogue Scale (VAS) is used by the child to measure pain experienced over the previous week: a 100 mm horizontal line with developmentally age appropriate descriptions at each end ("not hurting" or "no

pain" to "hurting a whole lot" or "severe pain") is provided, onto which the respondent makes a mark that they feel represents their pain, varying from 0 (no pain) to 10 (worst pain). The PPQ also has a gender-neutral body outline and four boxes beneath for descriptive categories of pain intensity. The child is given 8 colouring crayons and asked to select the colour that matches his / her pain intensity, colouring the 4 boxes with selected colours and then colouring in the body outline provided with the selected colour and intensity match. Children under 7 years of age may need assistance with completing the forms (Rapoff, 2003).

The body outline drawings may help the clinician in identifying specific areas of pain the child may not be able to verbalise, or that may not have been previously identified. Pain descriptors may be helpful in providing additional information to the clinician and potential differential diagnosis, and in combination with body outlines and the use of colours, can be used to measure the effectiveness of pain interventions (Walco et al., 1992). Much variability in the use of colours and body outline drawings was found in a study assessing musculoskeletal pain in 100 children with rheumatoid disease; there is however minimal evidence to suggest that specific interpretation can be made regarding pain using colour alone, but by finding what colour a child associates with pain severity, pain interventions can be assessed and consequently acted upon. The study by Varni et al (Varni et al., 1987) using the PPQ for assessing pain in children with rheumatoid arthritis, showed that the relationship between disease activity and present pain intensity using the Visual Analogue Scale (VAS) had construct validity. Furthermore, the PPQ was an effective method in measuring self-perceived pain by the child including the effectiveness of pain management interventions.

In 2011, the PPQ was recognised by the rheumatology community as a widely used and appropriate pain measurement tool for children with rheumatoid arthritis, compared to other pain measurements (Catrina et al., 2011) and that it was presented in a developmentally appropriate format. The authors suggest the PPQ is a well-established instrument for use in clinical and research

settings and therefore the PPQ questionnaire was selected for use in the current study.

The Pediatric Pain Questionnaire was used under license.

3.7.3.4. Piers- Harris Children's Self Concept Scale (PH2)

Among the self-esteem inventories, the Piers-Harris Children's Self Concept Scale (PH2) provides a validated self-report questionnaire for children aged 7-18 years and is used to measure self-esteem and how a child feels about himself / herself. The scale was originally developed from research based on what children liked and disliked about themselves and has been adapted through expert review and revalidation to represent the characteristics of USA populations through the use of 60 questions (Piers and Herzberg, 2002). The domains covered include behavioural adjustment, intellectual and school status, physical appearance and attributes, freedom from anxiety, popularity, happiness and satisfaction, with higher scores indicating higher self-esteem or self-regard.

Lack of physical activity in young people who are physically disabled can lead to obesity and reduced level of fitness, reduced self-esteem and a loss of inclusion in society (Wilson and Clayton, 2010). The authors suggest that participation in physical competitive sports as a young child leads to a positive outlook in life, including high self-esteem. The results from a large study of 8,491 children and adolescents between 10 and 19 years old suggested that self-concept and self-esteem are reduced in adolescents who are physically and / or developmentally disabled when compared to normative values (Ferro and Boyle, 2013a). The PH2 has been used to assess the self-esteem of children with spinabifida in a study of 329 children in two treatment centres in Canada, and found to provide useful information regarding the self-esteem of this group of children (Parkin et al., 1997).

The PH2 has been identified as an appropriate measure for assessing self-esteem in children with physical disabilities including spinabifida. It was therefore selected as an appropriate tool for the research group.

3.7.3.5. PAQ- C / PAQ-A (Physical Activity questionnaire)

Levels of physical activity are an important element in population surveillance and for policy makers with regards to health promotion and management (Bauman et al., 2006). Physical activity is linked to health outcomes and the participation of children in life activities is recognised as important in both clinical and psychological health (Voss et al., 2013).

In a review of articles published in 437 journals to determine which self-report instruments were the most suitable and feasible for use in Europe in measuring physical activity in children, the PAQ-C (for children aged 8-14) and PAQ-A (aged 14-20) were identified as being valid, objective, time efficient, easy to use and suitable for surveillance and monitoring (Biddle et al., 2011).

The self-administered scale measures weekly participation in sporting activities over the previous 7 days and categorise children into groups of those who are “sufficiently active” and “not at risk” (from ill health derived from inactivity) and those who are “low active” and “at risk. Voss and colleagues (Voss et al., 2013) studied 7,226 children and adolescents aged between 10 and 15 years old in the UK, and identified “no risk”, “at risk “and “high risk” children with regard to physical activity using the PAQ questionnaire; the authors concluded that the PAQ questionnaire provided standardised values that could be used in future studies.

Criticism of the scale includes the fact that it is designed for assessment only during the school year (Biddle et al., 2011). In addition, the authors identify that short and sporadic bursts of energy that younger children undertake are not accounted for in the scale. Despite these, the review suggests that the PAQ –A and PAQ-C provide an effective measure of physical activity that can be used in

designing interventions and understanding patterns and influences of behaviour. It was therefore selected as an appropriate tool for the research group.

The PAQ –C and PAQ –A were used under licence (The Physical Activity Questionnaire for Older Children (PAQ-C) and Adolescents (PAQ-A)

3.7.3.6. Discussions with the child

Tapia et al (Tapia et al., 2013) undertook a systematic review of patients with urinary dysfunction, which highlighted the importance of decreasing the most bothersome symptoms from the patients' perspective (in this case, urinary incontinence), in order to enhance their HRQL. To identify what were the issues of most importance to the children in terms of their disease which might not have been captured in the questionnaires, all children in the current study were asked to list the 5 most important of these issues and rank them in order of importance, with the most important listed first and the least important listed last.

The symptoms described by the children were documented and ranked in order of importance by the researcher, as described by the children. Overarching themes were then extracted from the data and entered onto a spreadsheet by the researcher, and ranked in order of importance. Key concepts that contribute to our understanding of what the most important issues are to children with LSL from the child's perspective, was thus captured.

3.7.4. Questionnaires completed by the parent / proxy

Questionnaires completed by the parent are displayed in table 3.4

Table 3.4 Questionnaires completed by the parent

Participant	Questionnaire	Time Taken to Complete *(In Minutes)*	Parameters Measured
Parent of child under 5 years	PedsQL PedsQL pain PIP HAD	5 5 15 5	Child's Quality of life Pain and sensory scoring Parenting stress Mood
Parent of child over 5 years	CHQ-PF50 PedsQL PedsQL pain PIP HAD	15 5 1 15 5	Child's Quality of life Child's Quality of life Pain and sensory changes Parenting stress Parent mood

3.7.4.1. The PedsQL 4.0 Generic parent score

Parent -proxy-report scales have been developed as generic core measures, are parallel to the child self-report forms and are designed to assess the parent's perceptions of their child's HRQL. Parent -proxy report includes ages 2–4, 5–7, 8–12 and 13–18. Although parent- proxy reports should be conducted with the knowledge that such ratings of HRQL may not be sufficiently accurate, they do provide data on which assessment and intervention can be based (Varni et al., 2002).

Analysis from parent -proxy report of 13,878 children using the PedsQL 4.0 parent score, demonstrated the reliability, validity and feasibility of the score (Varni et al., 2007b). Furthermore, the authors suggest that it is the parent-proxy evaluation of their child's HRQL that primarily influences health care

utilisation, and that this has important implications with regard to healthcare provision.

Eiser and Varni (Eiser and Varni, 2013) acknowledge the variability between child and parent-proxy reports of the child's HRQL and attempt to explain the circumstances in which these variations might happen. They suggest that the individual characteristics of the child and parent / proxy are relevant and that the impact of the disease may be viewed differently between child and parent / proxy. Both views are equally important and they suggest both are equally evaluated through use of the PedsQL.

The PedsQL generic parent score has been used in families of children with spinabifida. The PedsQL generic parent score and the PedsQL generic child score were sent to 172 children with spinabifida and their parents (Wide et al., 2014). The children were undertaking / receiving different bowel regimes (antegrade colonic enemas and transanal irrigation) and the study aim was to assess the HRQL of the child and parent. Although the children reported no significant difference between the two regimes, the parents reported a higher HRQL using antegrade colonic enemas and the authors suggest that children who were able to manage their bowel function independently from the parent (using the antegrade enema) resulted in a higher parental HRQL. The authors suggested the generic PedsQL was an appropriate tool for this group of children and families in identifying differences in HRQL elating to bowel management.

Freeman and colleagues (Freeman et al., 2013)assessed the HRQL of children with spinabifida and their parents with regard to bowel and bladder continence using the PedsQL. They identified no difference in scores with regard to continence but for the total score, parents scored their child's HRQL lower than the child's score. The authors found the PedsQL an appropriate measure for assessing the HRQL of children with spinabifida and their parents. Therefore, the PedsQL was identified as a suitable measure for the study group.

3.7.4.2. The Child Health Questionnaire (CHQ-PF50) parent / proxy

The CHQ-PF50 is a validated generic proxy measurement of HRQL completed by parents /carers of children and adolescents aged 4-18 years old. The shorter version of the child health parent questionnaire (CHQ-PF28) was not felt to provide sufficient detail for our research group and consequently the more detailed CHQ-PF50 was utilised (Raat et al., 2005). This consists of a 50-item parent report form that has physical and psychosocial scores based on 12 specific domains and two summary composite scores. It contains the same domains as the child form (CHQ-CF87) but includes an additional two domains by which to capture parental impact/time and parental impact/emotion. The questionnaire is based on a four week recall, takes 10-15 minutes to complete, and contains a range of 4-6 Likert type scales, from which a total score can be calculated. A higher score indicates a higher HRQL of the child.

Wang and colleagues (Wang et al., 2013) used the CHQ-PF50 to evaluate the HRQL of 32 children with myelomeningocele and 28 with lipomyelomeningocele and to determine any differences between the two groups. He found the CHQ-PF50 a useful measure by which to undertake the study and concluded that the detail provided by the questionnaire demonstrated that the children with the more severe disease (those with myelomeningocele) had a reduced HRQL than those with less severe disease (those with lipomyelomeningocele) but that the two groups had comparable psychosocial summary scores.

The CHQ -PF50 has been translated into several languages and is validated for use in the UK (Mulligan et al., 2013). The CHQ -PF50 has been an effective and validated tool for assessing the parents of 29 children with spinabifida (Rendeli et al., 2005) providing detail with regard to parental input and the effect of the child's disease on the family. The authors concluded that the multiperspective questionnaire provided useful information for this complex group of children and the CHQ-PF50 was consequently identified as appropriate for the research group.

3.7.4.3. Parent / proxy pain questionnaire (PPQ)

The PPQ proxy pain questionnaire is completed at the same time as the child completes his / her own PPQ questionnaire.

The importance of assessing parental/ proxy assessment of their child's pain is an important part of management of children and was studied within a hospital setting by Sherman and colleagues (Sherman et al., 2006). The authors studied the pain ratings of 70 children and the pain ratings of their parents and found the questionnaires had internal consistency reliability and construct validity and were an appropriate method of assessing a child's pain.

Significant variation has been found between the child's own perception of their chronic pain and the perception of their parents, with many confounding factors being suggested as factors in this variation, including the child's age and the HRQL of the parent (Vetter et al., 2012). The child's cognitive interpretation of pain may influence their own perception of pain, for example the wearing of a caliper, which is irritating and inconvenient, may be interpreted as pain by the child, but not so by the parent. While this discordance is important to recognise, it does not negate the importance of measuring both the child and parent's perception of the child's pain, in forming a holistic picture.

3.7.4.4. The Hospital Depression and Anxiety Scale (HAD).

The HAD was Developed in 1983 to measure levels of depression and anxiety in a hospital outpatient setting (Zigmond and Snaith, 1983) and has since been widely used in studies of adult patients (McCairn and Jones, 2014), adolescents and young adults (Taylor et al., 2013) and parents of sick children (Ben Thabet et al., 2013).

This 14-item self-assessment questionnaire is scored on a Likert scale of 0 (not present) to 3 (considerable) with a resulting score of between 0-21 for either depression or anxiety. A literature review of 747 papers using the HAD scale demonstrated a similar level of sensitivity and specificity to those used in other

general health questionnaires, and was found to be effective in assessing depression and anxiety disorders in the general population as well as in patients (Bjelland et al., 2002).

Mothers of disabled children may be more at risk from depression and anxiety than mothers of healthy children, with the level of depression and anxiety rising with the more disabled and dependent child (Al-Eithan et al., 2013). Al-Eithan et al identified the Hospital and Depression Scale (HAD) as an appropriate measure by which to assess 86 mothers of disabled children when compared with 32 mothers of healthy children and did not identify any correlation between demographics, maternal age and the level of depression and anxiety.

In a study by Ben Thabet (Ben Thabet et al., 2013) the effect of parenting a child with a disability was assessed using the HAD scale; the study results identified a higher rate of anxiety and depression amongst mothers of disabled children, but the authors acknowledged other potential confounding effects on quality of life including, socio economic issues and variety of coping strategies.

It has not been investigated to date, if parents of children with LSL have a greater level of depression and anxiety than parents of healthy children. The HAD scale was therefore included in the research study, to ascertain if parents of children with LSL had a greater level of depression and anxiety than a control group of parents with healthy children.

3.7.4.5. The Pediatric Inventory for Parents (PIP)

The Pediatric Inventory for Parents (PIP) was developed within the Oncology department at the Children's Hospital of Philadelphia. The PIP provides a measure of parenting stress related to caring for a child with a chronic illness, with information of the experience of the parent that is most relevant to their child’s illness. It has been recognised as particularly pertinent for use where a chronic disease is intermittent and unpredictable, and has been shown to have excellent internal consistency, reliability and validity (Gray et al., 2013, Streisand et al., 2001).

The scale consists of 42 items, grouped into four domain scales. Each item is scored by the parent in terms of difficulty and frequency experienced during the previous week, on a Likert scale of 1-5. A higher total score indicates a higher frequency and difficulty of the item. Examining responses on the PIP to assess disease severity, parenting stress, family functioning and emotional functioning, can guide the need for psychological interventions and support.

Parenting a child with a chronic illness requires balancing family responsibilities and tasks, finances and jobs, with caring for the sick child (Guilfoyle et al., 2012). The author concludes from their study of 62 adolescents with chronic bowel disease, that the needs of carers must be recognised in addition to the needs of the sick child, and that the Pediatric Inventory for Parents (PIP) provided an appropriate method of providing this information.

Little is known about the parental stress experienced by parents of children with LSL and this validated scale was therefore adopted for the study to provide this information and guide potential interventions.

3.7.4.6. Discussions with the parent.

The parents were asked in a similar method to the children, to rank in order of importance what was most important to them in terms of their child's disease. Data was collected and documented in a similar way to that described in the discussions with children section.

3.8. Procedures

A single researcher conducted the study. Children and parents were given the option to complete their questionnaires at home or when they came to the clinic; many of them selected the latter, as they had a lengthy wait between their morning appointment with urodynamics and their afternoon appointment with the neurosurgeon. Questionnaire packs were arranged in random order to prevent systematic bias. Children and parents were told of the importance of

completing questionnaires on their own, without help from another person apart from the researcher.

The chart produced by the researcher to measure the clinical symptoms associated with LSL was completed in the clinic setting.

3.8.1. Pilot study

A pilot study was undertaken to identify if the procedure for obtaining assent and consent was appropriate, if the children and parents understood how to complete the questionnaires and if the questionnaires selected, were the most appropriate in answering the research questions.

3.8.1.1. Pilot sample

The first five children and their parents recruited into the study comprised the pilot study, consisting of 3 girls and two boys of variable ages and ethnic orientation. The first children recruited undertook the GAITRite assessment (see 3.8.1.3.1). They were of variable ages and ethnic orientations.

3.8.1.2. Pilot procedure

Recruitment to the pilot study followed the study procedure as previously described. All five children and parents chose to complete the questionnaires in the clinic setting rather than at home. All questionnaires were complete in full by the child and parent. In addition to routine clinical assessments as described previously, 18 children were assessed using the GAITRite. The physiotherapist undertook the GAITRite assessments and the remaining clinical assessments were undertaken by the neurosurgeon in the outpatient setting

3.8.1.3. Pilot results

All questionnaires and clinical assessments were undertaken within the timeframe of the outpatient clinics and the children and families found this acceptable. On talking with the families and children, it became clear that as the

questionnaires were generic, information specific to the disease and seen as important to the families and children was not being obtained. As there are no questionnaires appropriate for our participant group, participants (parents and children) were asked to describe the five most important aspects of the disease, ranked in order of importance to them, with the most important scored as one and the least as 5. All information was treated as confidential in the same manner as the questionnaires and anonymised.

Clinical assessments and results from the GAITRite were documented.

3.8.1.3.1. The GAITRite

The GAITRite was used to describe gait parameters including velocity and cadence and identify any asymmetry or difficulty in walking and the data was interpreted against normative data. 18 children were recruited into a pilot study to assess if the GAITRite walkway was a useful assessment tool for our research group of children with LSL in identifying gait changes. A one sided t test was used to compare the z scores from the normal mean.

There was no statistical significance between the normative data collected from 625 typically developing children aged 1-18yrs and the data from the children with LSL. All 18 children with LSL fall between the 10th and 97th centiles, with a mean Z score -0.0.4 and p value of 0.82.

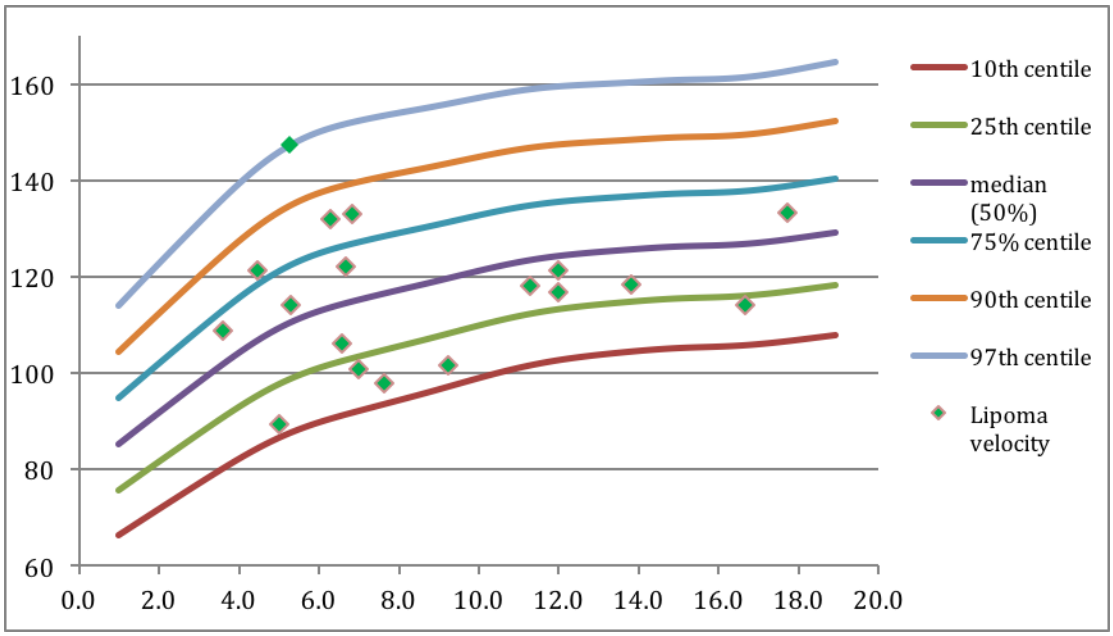


Figure 3.1 GAITrite analysis of 18 children with LSL

3.8.1.4. Review and actions taken following pilot study

A review of the procedure to facilitate posting the questionnaires to the families was submitted and accepted by the REC committee. Families were then given the option of being sent the questionnaires by post in advance of their outpatient appointment if this was more convenient to them or completing them between appointments. If they chose the former, they would be given the opportunity to ask questions to the researcher, prior to signing consent / assent forms and completing the questionnaires.

While the GAITRite provided useful data, it proved time consuming to undertake in the clinical outpatient setting, required space in which to undertake the assessments and required complex analysis of the data, with additional time burden. The tool was identified as useful in monitoring changes as the child grows, especially for children with more complex LSL and neuro-orthopaedic presentation. The GAITRite was impractical for use in the busy study setting but should be considered for future research.

3.9. Statistical analysis of research data.

Statistical analyses were undertaken using IBM SPSS Statistics Version 21. The analysis proceeded in four stages, each building to some extent on the preceding stages.

The first stage focused on medical outcomes. The second stage focused on pain and physical activity. The third stage focused on more holistic child-centred psychological outcomes, including measures of HRQL and self-esteem. Finally, the fourth stage focused on parent- centred outcomes, including mood and experience of paediatric illness.

All tests of statistical significance were 2 tailed ($p \leq 0.05$). Given the exploratory nature of the study, no correction for multiple comparisons was applied as given the number of comparisons, the results would be too small to be significant.

3.9.1. Medical outcomes

The process of analysis of each variable followed essentially the same sequence of steps.

First, the distribution of the outcome variable was described. For categorical variables, this was in terms of frequencies. For ordinal variables, medians were preferred.

Second, for multicomponent measures, the correlations between the different components were evaluated. Given that the measures did not meet assumptions for parametric statistics, Spearman's Rank Order Correlations were reported.

Finally, the outcome variable was examined for group differences by gender, LSL type and presence/absence of a syrinx. For categorical outcome variables, given the likelihood of expected cell counts of less than 5 in at least some contingency tables, Fisher's Exact Test was used. For ordinal outcome data, the effects of gender and presence/absence of a syrinx were tested using the

Mann-Whitney U Test. The effect of LSL type was tested using the Kruskal-Wallis Test, with post hoc Mann-Whitney U test if indicated. For some group comparisons, mean ranks were reported rather than medians if medians were tied.

3.9.1.1. Pain and physical activity

The analysis of pain and physical activity data followed the same steps as for Medical outcomes.

In addition, the relationships between pain, physical activity and medical outcomes in terms of the NEM scales, were evaluated through Spearman's Rank Order Correlations.

3.9.1.2. Child-centred psychological outcomes

The description of these variables included the Kolmogorov-Smirnov test of normality. For the majority of measures, this indicated that the data did not conform to a normal distribution. For this reason, non-parametric statistics were preferred.

Where possible, one sample Wilcoxon tests were used to compare the obtained medians with estimates from published normative values for healthy participants or the general population. Typically, the normative values were reported in terms of means and standard deviation. Consequently, the reported mean was used as an estimate of the median. Given the possibility that this estimate might be inaccurate, one-sample t-tests were also performed "as if" the data justified parametric tests. These are reported in the appendices.

From this step onward, the analysis of child-centred psychological outcomes followed the same steps as for the preceding stage, including the effect of medical outcomes.

In addition, analyses considered the influence of pain and physical activity though Spearman's correlations.

3.9.1.3. Parent-centred psychological outcomes

The analysis of parent-centred psychological outcomes data was undertaken using similar same steps to that used for the child-centred outcomes.

Comparison with normative data was not possible for one of the measures (PIP) as it is specifically a measure of child illness-related parenting stress.

3.10. Summary

Following data collection as outlined in this chapter, the clinical and HRQL results as discussed in the following chapters and the salient assessment criteria highlighted in the systematic review, preliminary steps towards the development of a clinician's assessment tool were undertaken. The resulting assessment tool is provided in chapter 8; validation, acceptability and feasibility of the tool are required and will be addressed in post doctorate research.

The following chapter will provide the results of the clinical examination and variables and the PAQ Activity scale.

Chapter 4. Clinical variables

4.1. Demographics

In the study centre there are 150 children with lumbosacral lipoma (LSL) and approximately 8-10 are seen per month, which includes both new patients and review of existing patients. Excluding children with complex co morbidities such as cloacal atresia and children under five years of age in line with the study protocol discussed in chapter 3, it was anticipated that over the data collection period of 18 months, 54 children and their parents would be recruited. Between January 2012 to July 2013, 54 parents and 54 children were recruited and all children underwent routine clinical assessment during their outpatient appointment. All participated in returning questionnaires, which were completed in full. A further 3 families did not wish to participate due to personal circumstances: One family felt as their child was currently asymptomatic they did not wish her to know there was a potential for her to deteriorate, a second family gave no reason and the third family felt their lives were too busy to participate and did not wish to be approached at a further date. All participants were under the care of the neurosurgical department at Great Ormond Street Hospital for Children, NHS Foundation Trust, London, and the children were aged between 5-18 years of age with a diagnosis of LSL.

Demographic details were taken from the parent who attended the appointment with the child and this was consistently the mother, which may reflect childcare requirements. The majority of parents were between 31-35 years old, married or with an in- house partner and 35.5% were not currently working (as defined by unemployed / stay at home / retired). Parents' occupations were categorised using the Office for National Statistics, standard occupation classification (Office for National Statistics, 2010). Table 4.1 presents parent demographics and table 4.2 child demographics.

Table 4.1 Parent demographics

Participating parent n: 54	Percentage	Number
Gender		
Mother	100	54
Father	0	0
Age		
26-30	14.8	8
31-35	62.9	34
36-40	12.9	7
>40	9.2	5
Marital status		
Married / in-house partner	94.4	51
Single / divorced / separated	5.5	3
Ethnicity		
White British	66.6	36
Black British	5.5	3
Asian British	25.9	14
Other	1.8	1
Education level		
Secondary School	79.6	43
Educated abroad	9.2	5
Graduate	11.1	6
Post graduate	0	0
Occupation		
Managers, directors and senior officials	3.7	2
Professional occupations	22.2	12

Associate and professional and technical occupations	0	0
Administration and secretarial occupations	25.9	14
Skilled trades occupations	0	0
Caring, leisure and other service occupations	11.1	6
Sales and customer service occupations	25.9	14
Process, plant and machine operatives	0	0
Elementary occupations	0	0
Unemployed / stay at home parent / retired	11.1	6

Table 4.2 Child demographics

Participating child n: 54	Percentage	Number
Age		
5-7 years old	35.1	19
8-12 years old	31.4	17
13-18 years old	33.3	18
Ethnicity		
White British	66.6	36
Black British	5.5	3
Asian British	27.7	15
Other		
Gender		
Female	66.6	36
Male	33.3	18

The ethnicity of children and parents was recorded and showed a small increase in the White British participants compared to the published data for ethnicity for London, whereby 45% of London's population are White British and 7% of Indian and Black African origins (Trust for London, 2013). The hospital is a tertiary referral hospital with a small number of children living outside London.

4.2. Medical Variables

4.2.1. Sample diagnostics

Table 4.3 describes 3 LSL groups, in terms of gender (number and percentage) and age (median and interquartile range).

Table 4.3 Description of gender

Interquartile range*

Description of gender (number and percentage) and age (median and interquartile range) of 3 LSL groups									
		Caudal		Dorsal		Transitional		Total	
		n	%	n	%	n	%	n	%
Gender	Female	13	24.1	12	22.2	11	20.4	36	66.7
	Male	6	11.1	3	5.6	9	16.7	18	33.3
Total		19	35.2	15	27.8	20	37	54	
		Median	IQR*	Median	IQR*	Median	IQR*		
Age (years)		10	5	10	6	8	8		

4.2.1.1. LSL type and Gender

There were significantly more females than males in the whole sample (66.7%; Binomial test p = 0.02). Females were also in the majority for each of the LSL types; statistically significantly so for the dorsal (p=0.04), but not caudal (p=0.17) or transitional LSL (p=0.82) groups.

There was no significant association found between LSL type and gender (p=0.34, Fisher's Exact test).

4.2.1.2. LSL type and Age

Age was normally distributed in the caudal and dorsal groups ($p \geq 0.20$ for both, Kolmogorov-Smirnov test), but not the transitional group (p=0.03). Therefore, medians and interquartile ranges are reported which showed that the caudal and dorsal groups tended to be older than the transitional group, although the differences were not statistically significant (p=0.57, Kruskal-Wallis Test).

4.2.1.3. Gender, the presence of a Syrinx, and LSL Type

A syrinx is an intraspinal fluid-filled cavity, which has previously been suggested to be a poor prognostic feature (Wykes et al., 2012). A syrinx was identified on Magnetic Resonance Imaging scans (MRI) as reported by an independent radiologist in 16 of the 54 patients (29.6%).

Divided by gender, 27.8% of females and 33.3% of males had a syrinx, this difference being statistically insignificant (p=0.756).

Of the three LSL groups, syrinxes were more common in those with transitional lipomas (45%) than either caudal or dorsal lipomas (21.1% and 20% respectively). However, differences were not statistically significant (p=0.19).

4.2.1.4. Summary of sample demographics

Females were in the majority in each of the 3 LSL groups and statistically, significantly outnumbered males in the dorsal group and the total sample.

Age differences between genders and LSL types were not statistically significant.

Syrinxes were present to a similar extent in females and males. They were more than twice as common in transitional as other LSLs, but this difference was not statistically significant.

4.3. Clinical Outcome Variables

Each of the following variables were assessed using the following criteria: Neurology, pain, urology, bowel function and orthopaedic function. They were also classified according to the use or not, of Clean Intermittent Catheterisation (CIC). As described in the methodology chapter, children were described as normal (i.e. with no clinical deficits), children who had deficits but these were stable deficits (abnormal stable) and children who were clinically deteriorating. The prevalence of different outcomes is shown in figure 4.1.

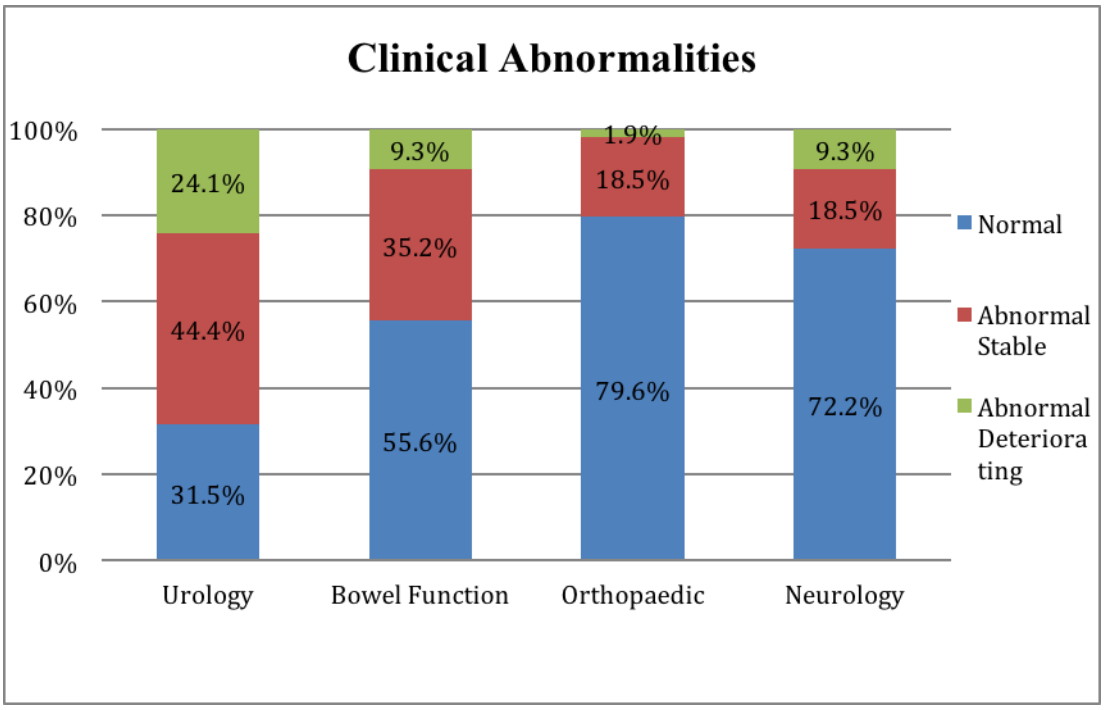


Figure 4.1 Percentage of normal, abnormal & deteriorating clinical outcomes in whole sample (n=54)

The x axis displays the 4 clinical variables and the y axis the percentage children who have normal, abnormal stable and deteriorating function within each of the clinical variables.

The results indicate that urological abnormalities were the most common symptom and present in 37 children (68.5%), with 24 children (44.4%) of the total number of children using Clean Intermittent Catheterisation (CIC). Deterioration in urological function was identified in more than a third of the children with urological abnormalities (13 children, 24.1% of the whole sample).

Other kinds of abnormality occurred in a minority of the sample, but still at clinically significant levels. Of these, orthopaedic abnormalities were the least common but present in 11 children (20.4% of the whole sample) with 1 child (1.85%) deteriorating.

For all clinical outcome variables apart from CIC, analysis proceeded in two steps. First, patients' status was classified and analysed as a binary variable: normal or abnormal (the latter group including both stable abnormalities and deteriorating abnormalities). Then outcomes were reclassified according to

another binary variable: stable or deteriorating (the former including both normal and stable-but-abnormal conditions).

These outcomes were examined for group differences by gender, LSL type and the presence/absence of a syrinx.

Given the likelihood of expected cell counts of less than 5 in at least some contingency tables, Fisher's Exact test was used for all tests for independence between clinical outcome variables and the other variables.

4.3.1. Categorical clinical outcomes and Gender

4.3.1.1. Neurology and Gender

Abnormal neurological signs were similarly prevalent in males and females (22.2% and 30.6% respectively; p=0.748, Fisher's exact test.

Of the 5 cases (9.3% of the whole sample) who had neurological deterioration, all were female. However, the effect of gender (13.9% of females compared to 0% of males) was not significant (p=0.16).

4.3.1.2. Urology and Gender

Urological abnormalities were present to a similar extent in females and males (66.7% compared to 72.2%; p=0.763). Furthermore, approximately equivalent proportions of females and males had deteriorated (25% and 22.2% respectively; p=1.00).

4.3.1.2.1. CIC and Gender

The association between gender and CIC was not statistically significant (38.9% of females and 55.6% of males; p=0.384).

4.3.1.3. Bowel Function and Gender

Bowel abnormalities of any kind were similarly common in females and males (47.2% and 38.9% respectively; p=0.77), as were deteriorating bowel function (11.1% of females; 5.6% males; p=0.655).

4.3.1.4. Orthopaedic Function and Gender

Orthopaedic abnormalities were present in statistically similar proportions of females (19.4%) and males (22.2%) (p=1.00).

4.3.1.5. Clinical outcomes and Gender: Summary

Gender did not appear to exert a significant effect on categorical clinical outcomes.

4.4. Clinical outcomes and LSL type.

All clinical outcomes present a snapshot view of the child's status at a given moment in time and as such, present a limitation to the study.

The frequency of abnormal clinical findings in different LSL groups is shown in figure 4.2. This depicts a trend towards cases of transitional lipomas being more affected in most respects than cases of caudal and particularly dorsal lipomas.

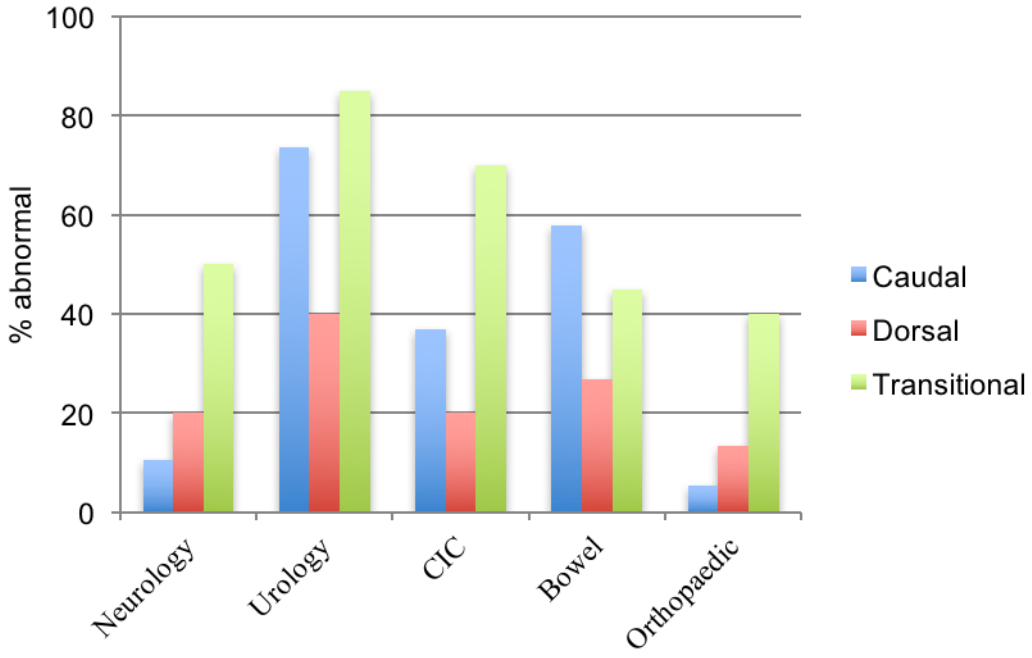


Figure 4.2 Frequency of abnormal clinical findings in 3 LSL groups

The x axis displays the clinical variables and the y axis the percentage of children who have an abnormality of those variables.

4.4.1. Neurology and LSL type

There was a significant association between LSL and abnormal neurology (p=0.02). There was a higher incidence of abnormal neurology in the transitional lipoma group (50%) than in the dorsal (20%) or caudal groups (10.5%).

A higher proportion of the transitional lipoma cases were deteriorating (15.0%) than the dorsal (6.7%) or caudal (5.3%) cases. However, the number of deteriorating cases in each LSL group was low and the specific association between lipoma type and neurological deterioration (i.e. compared to a combined normal/stable category) did not approach statistical significance (p=0.61).

4.4.2. Urology and LSL type

There was a significant association between LSL type and abnormal urology (p=0.02). The majority of cases of transitional and caudal lipoma had urological abnormality (85% and 73.7% respectively), whereas only a minority of the dorsal lipoma group was affected (40%).

Urological deterioration was apparent in the minority of each lipoma group (30% of transitional; 13.3% of dorsal; 26.3% of caudal), with no specific association between lipoma type and deterioration (p=0.57).

4.4.2.1. CIC and LSL type

There was a highly significant association between LSL type and CIC (p=0.01). This procedure was most used by the transitional group (70%), and less frequently by the caudal (36.8%) and dorsal (20%) group.

4.4.3. Bowel Function and LSL type

Bowel abnormalities were apparent in all 3 LSL groups (57.9% of caudal cases; 45.0% transitional; 26.7% dorsal; p=0.19).

Whereas no participants with dorsal lipomas followed a deteriorating course, 15% of transitional cases and 10.5% of caudal cases had deteriorated. However, the association between LSL type and the presence of deteriorating bowel function was not statistically significant (p=0.42).

4.4.4. Orthopaedic Function and LSL type

Orthopaedic abnormalities were present in a minority of cases in all 3 LSL groups.

Even so, there was a significant association between LSL type and orthopaedic abnormalities (p=0.03). These abnormalities occurred more frequently in the

transitional group (40.0%) than the dorsal group (13.3%), and occurred least frequently in the caudal group (5.3%).

Only one case involved orthopaedic deterioration, which was associated with a transitional lipoma.

4.4.5. Clinical outcomes and LSL type: Summary

There were significant effects of LSL type on the presence of abnormal neurological, urological, CIC, and orthopaedic outcomes. In each case, outcomes of transitional lipomas were poorer than either caudal or dorsal lipomas.

Group differences in bowel abnormalities were not significant.

Cases following a deteriorating course were relatively rare and there was no statistical significance between clinical deterioration and LSL type.

4.5. Clinical outcomes and the presence of a syrinx

Rates of abnormal findings in patients with and without a syrinx are shown in figure 4.3.

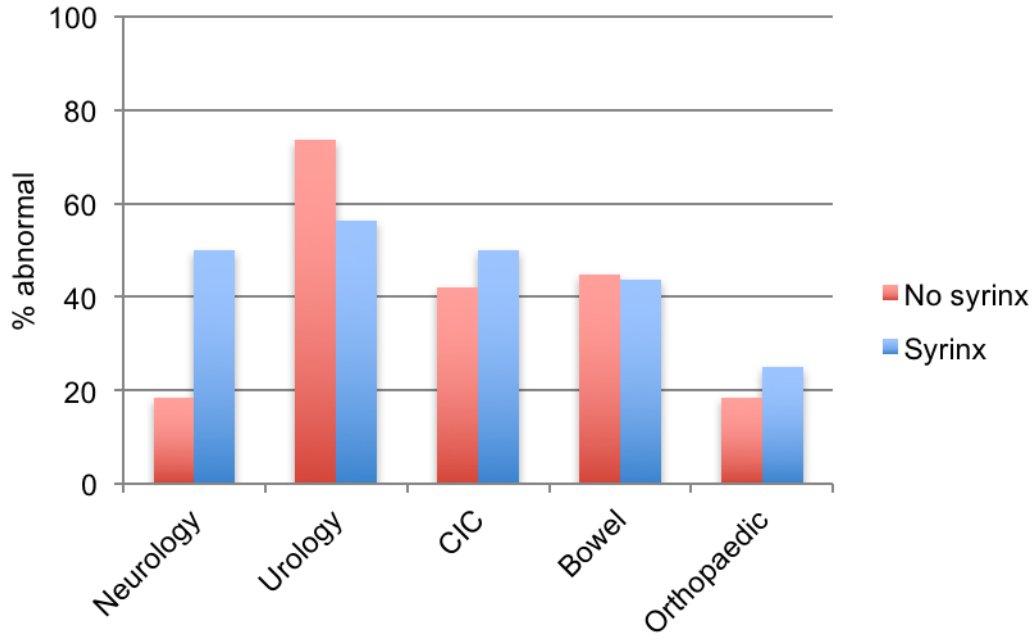


Figure 4.3 Frequency of abnormal clinical findings by presence/ absence of a syrinx
The x axis displays the clinical variables and the y axis displays the percentage of each of the clinical variables in the presence / absence of a syrinx.

4.5.1. Neurology and the presence of a syrinx

Abnormal neurology (either stable or deteriorating) was present in 18.4% of those without a syrinx, but 50% of those with one. This difference was statistically significant (p = 0.04).

However, the association between presence of a syrinx and neurological deterioration was not statistically significant (p=0.15).

4.5.2. Urology and the presence of a syrinx

The presence of a syrinx was not significantly associated with either abnormal urology (p=0.22) or specifically urological deterioration (p=0.732).

4.5.3. CIC and the presence of a syrinx

There was no significant association between the presence of a syrinx and CIC (p=0.765).

4.5.4. Bowel Function and the presence of a syrinx

The associations between the presence of a syrinx and bowel abnormalities or bowel deterioration were not significant (p=1.00 and p=0.63 respectively).

4.5.5. Orthopaedic Function and the presence of a syrinx

The association between presence of a syrinx and orthopaedic abnormalities did not approach statistical significance (p = 0.71).

4.5.6. Summary of clinical outcomes and the presence of a syrinx.

The presence of a syrinx was not found to be risk factor for most of the categorical clinical outcome variables with the exception of an association between abnormal (but not deteriorating) neurology.

In this context, the presence of a syrinx was specifically associated with a significantly increased risk of neurological abnormalities.

4.6. Necker-Enfants Malades ratings

In addition to the categorical clinical ratings described and analysed above, ratings on the NEM scales were collected (previously described in the methodology chapter; the NEM scale is provided in appendix 4.1. These include separate ratings of the degree of motor, sensory, urological and bowel function, which may be summed to calculate a Total score. Higher scores indicate a higher level of functioning (Kulkarni et al., 2004c, Pierre-Kahn et al., 1997).

The distributions of scores on the 4 subscales are shown in figure 4.4.

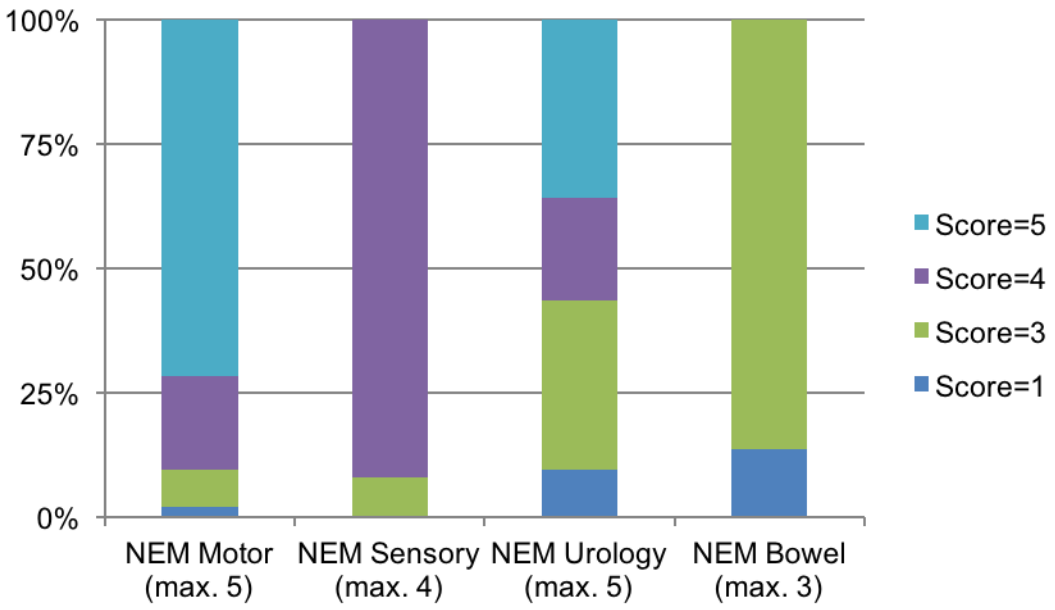


Figure 4.4 Distribution of scores on NEM subscales

The x axis displays the 4 domains of the NEM scale, the y axis the percentage obtained in each of the domain scores. The NEM motor score has a maximum (depicted as "max" on the table) score of 5, sensory 4, urology 5 and bowel function 3. A degree of at least mild urological dysfunction was present in the majority. However, the lowest ratings (indicative of poor function) were relatively uncommon on all scales.

As the data are not on an interval scale or normally distributed, non-parametric analyses were performed. Correlations between the NEM ratings showed that other than statistically significant correlations with the Total score (p=0.002), there were not significant relationships between the ratings.

With regard to the clinical outcomes, NEM ratings were examined for group differences by gender, LSL type and presence/absence of a syrinx.

4.6.1. NEM ratings and Gender

The analyses of NEM ratings by gender show that although females were in the majority, there was no statistically significant effect of gender on any of the NEM ratings.

4.6.2. NEM ratings and LSL type

Mean ranks for NEM ratings by LSL show group difference in NEM scores by LSL type as shown in figure 4.5. Mean ranks are presented rather than medians as medians were tied on several scales.

Inspection of this figure shows that cases of dorsal LSL tended to be ranked highest for 3 of the 4 NEM domains (Sensory, Urology, Bowel) and the lowest in none. In contrast, transitional cases tended to be ranked lowest for 3 of 4 domains (Motor, Sensory, Urology) and the highest in none. In this context, caudal cases tended to receive intermediate rankings, but were rated more highly than dorsal cases in Motor function and lower than transitional cases in Bowel function.

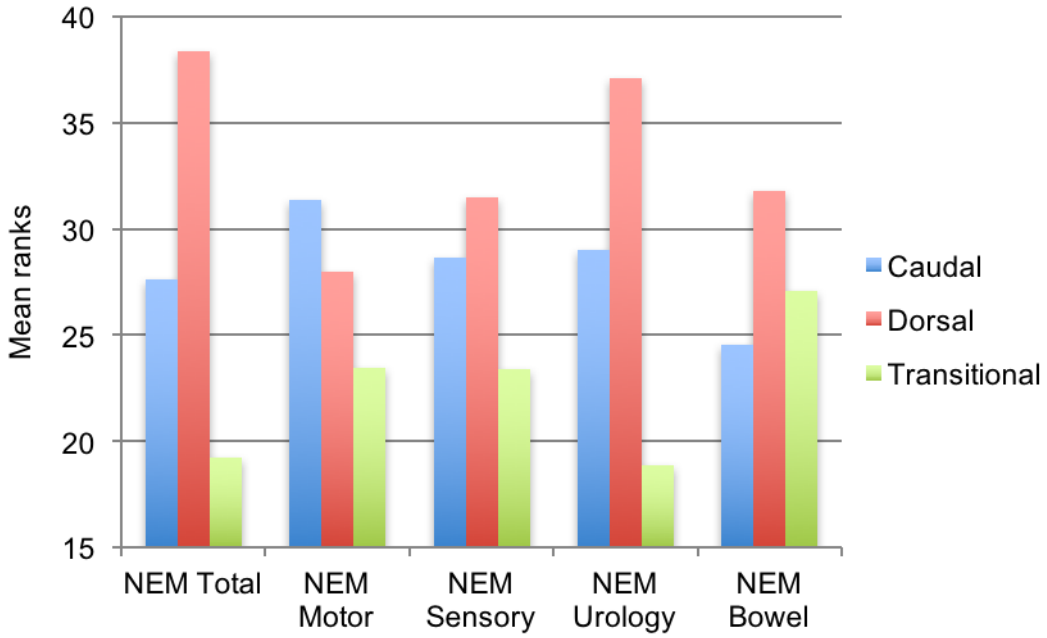


Figure 4.5 Mean ranks of NEM ratings by LSL type

The x axis displays the NEM domains, the y axis the mean ranks. The transitional group tend to rank lowest in most domains and the dorsal group the highest.

4.6.3. NEM Total and LSL type

A Kruskal-Wallis Test showed a statistically significant difference in Total NEM ratings across the 3 lipoma groups (p=0.001). Post-hoc Mann-Whitney U Tests indicated that dorsal lipoma group were ranked more highly than both the transitional and caudal group (p<0.001 and p=0.03 respectively). The transitional group tended to have higher NEM ratings than caudal cases, but this did not reach a statistically significant difference (p=0.08).

4.6.4. NEM Motor and LSL type

There were no significant differences between the 3 lipoma groups on the NEM Motor scale (p=0.15, Kruskal-Wallis Test).

4.6.5. NEM Sensory and LSL type

There was a statistically significant difference in NEM Sensory ratings across the 3 lipoma groups (p=0.04, Kruskal-Wallis Test).

This was attributable to a significant difference between the transitional and dorsal LSL groups (p =0.02, post-hoc Mann-Whitney U Tests), the latter showing fewer sensory abnormalities. Rankings of caudal and dorsal lipomas were not significantly different (p =0.202). Similarly, caudal and transitional lipomas did not differ significantly (p = 0.14).

4.6.6. NEM Urology and LSL type

There was a statistically significant difference in NEM Urology ratings across the 3 lipoma groups (p=0.002, Kruskal-Wallis Test). Transitional lipomas were rated lower than both dorsal (p>0.001, post-hoc Mann-Whitney U Tests) and caudal lipomas (p=0.04). Dorsal and caudal lipomas did not differ significantly in this measure (p=0.125).

4.6.7. NEM Bowel function and LSL type

Differences between NEM Bowel ratings for the 3 lipoma types were not statistically significant (p=0.25, Kruskal-Wallis Test).

4.6.8. NEM ratings and LSL type: Summary

Analysis of categorical clinical outcomes indicated significant effects of LSL type on the presence of abnormal neurological, urological and orthopaedic signs. In each case, outcomes of transitional lipomas were poorer than either caudal or dorsal lipomas.

Dorsal LSLs tended to have the least impact on function. Inspection of trends suggested that cases of dorsal LSL tended to be ranked the highest for 3 of the 4 NEM domains (including sensory and urological function) and the lowest in none. In contrast, transitional cases tended to be ranked lowest for 3 domains and highest in none, suggesting the greatest impact on function. In this context, caudal cases tended to receive intermediate rankings. The NEM total scores indicated that the dorsal group was ranked higher than either the caudal or the transitional groups, which were not significantly different from one other.

Further analyses indicated that the dorsal group was ranked significantly higher than the transitional group in the sensory and urological function. The caudal group was not clearly distinguished from either the dorsal or the transitional group in the sensory domain, but more similar to the dorsal than the transitional group with respect to urology.

None of the LSL group differences in NEM motor or bowel function were statistically significant.

4.6.9. NEM ratings and Syrinx

Mean ranks of NEM ratings by the presence/absence of syrinx are shown in figure 4.6. The presence of a syrinx tended to be associated with lower ranking in motor, sensory, and bowel functioning, but not with urological functioning.

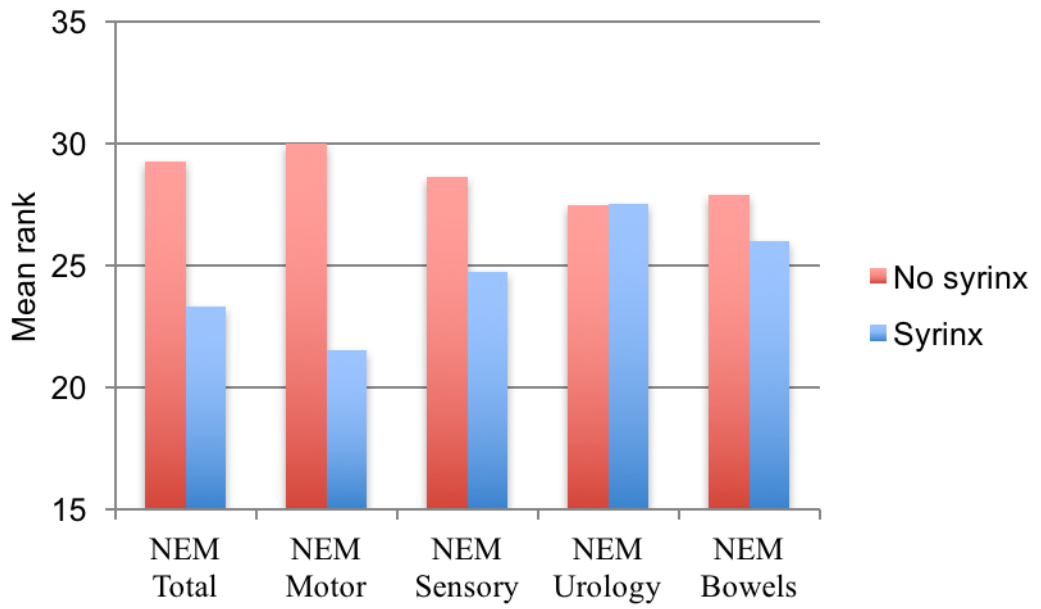


Figure 4.6 Mean ranks of NEM ratings by presence/ absence of syrinx

The x axis displays the NEM domains, the y axis the mean rank of each of the domains in the presence / absence of a syrinx.

NEM ratings of motor function were significantly higher for cases without a syrinx compared to those with a syrinx (p=0.02, Post-hoc Mann-Whitney U Tests). No other comparisons demonstrated statistically significant differences.

4.7. Pain

As described in the Methodology chapter, the study included several measures of pain.

4.7.1. Categorical rating of pain and location of pain

Participants were asked if they had any back or leg pain / both areas/ no pain and their response documented by the researcher. Over half the sample (51.9%) reported experiencing back and / or leg pain; leg pain was significantly more common than leg-and-back pain (75% compared to 25%; Kruskal-Wallis Test, p=0.008).

It was notable that no one reported back pain only.

4.7.2. Categorical pain ratings and Gender

There was no significant effect of gender on either binary reporting of the presence/absence of pain (p=1.00) or, in the location of pain for those reporting pain (p=1.00).

4.7.3. Categorical pain ratings and LSL type

There were 52.6% of patients with caudal lipomas, 33.3% with dorsal lipomas, and 60% with transitional lipomas who reported experiencing leg/back pain. Differences were not statistically significant (p=0.18).

Leg pain was more common than leg-and-back pain in all 3 groups, and no cases of dorsal LSL reported leg-and-back pain.

However, within the current sample sizes, LSL type was not significantly associated with specific locations of pain (p=0.352). The results are displayed in figure 4.7.

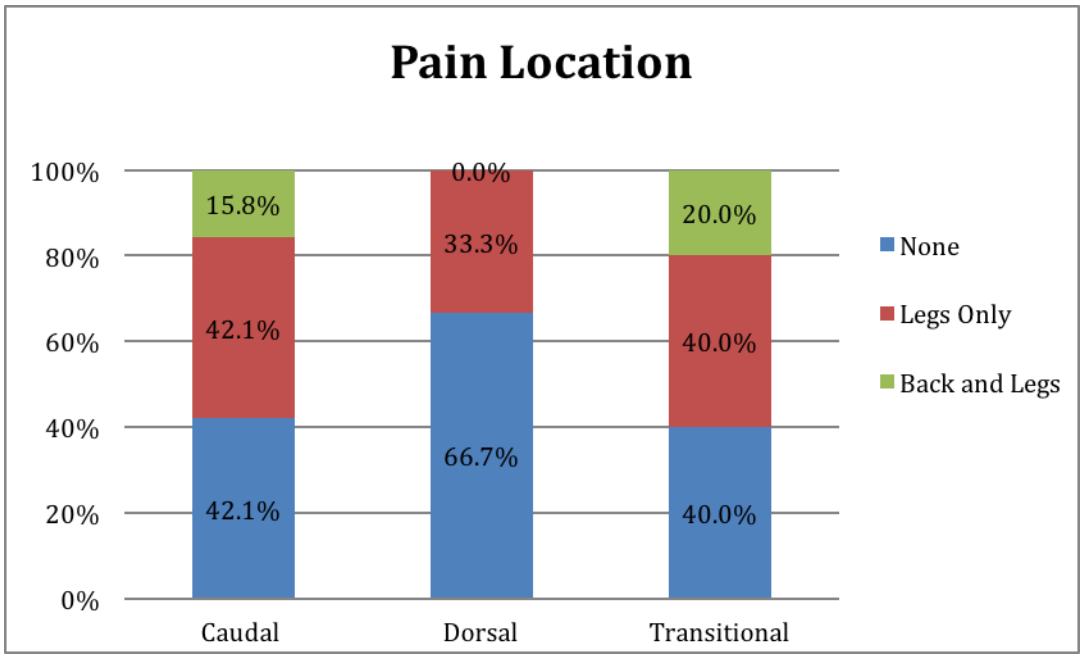


Figure 4.7 Location of Pain by Lipoma Type

The x axis displays the lipoma type, the y axis the percentage who have pain in legs only, back and legs, or no pain.

Proportionately more participants without a syrinx experienced pain than those with one, but this difference was not statistically significant (p=0.55).

4.7.4. Categorical pain: Summary

Pain was reported by approximately half of the sample and in all 3 LSL groups, most commonly in the legs, but also in both the leg-and-back for some patients with caudal or transitional LSL.

However, there were no statistically significant differences in reported pain by gender, LSL type, or presence of a syrinx.

4.7.5. Varni/Thompson Pediatric Pain Questionnaire (PPQ)

Both patients and their parents reported experience of pain on the Varni/Thompson Pediatric Pain Questionnaire (PPQ; Varni & Thompson, 1985). This included location and severity of pain on a "body map", and severity of present pain and worst-pain-in-the preceding week on visual analogue scales, which had a rating scale of 0-10. Values were rounded up to the nearest whole number.

4.7.5.1. Colour coding of pain on the PPQ body map

There were 25 children (46%) who completed the body maps using either colour coding and / or marking with a cross to indicate pain. All 25 children indicated pain in the legs and/or back-and-legs. One coloured in the head and legs, indicating headaches in addition to leg pain.

There were 18 children (33.3%) who used colour coding to complete the body map when describing pain on the PPQ chart as shown in figure 4.8 There were some trends in colour choice, notably white as signifying the absence of pain (the body map being left blank i.e. white) and red signifying severe pain. Red was also used however to depict moderate pain, as was blue.

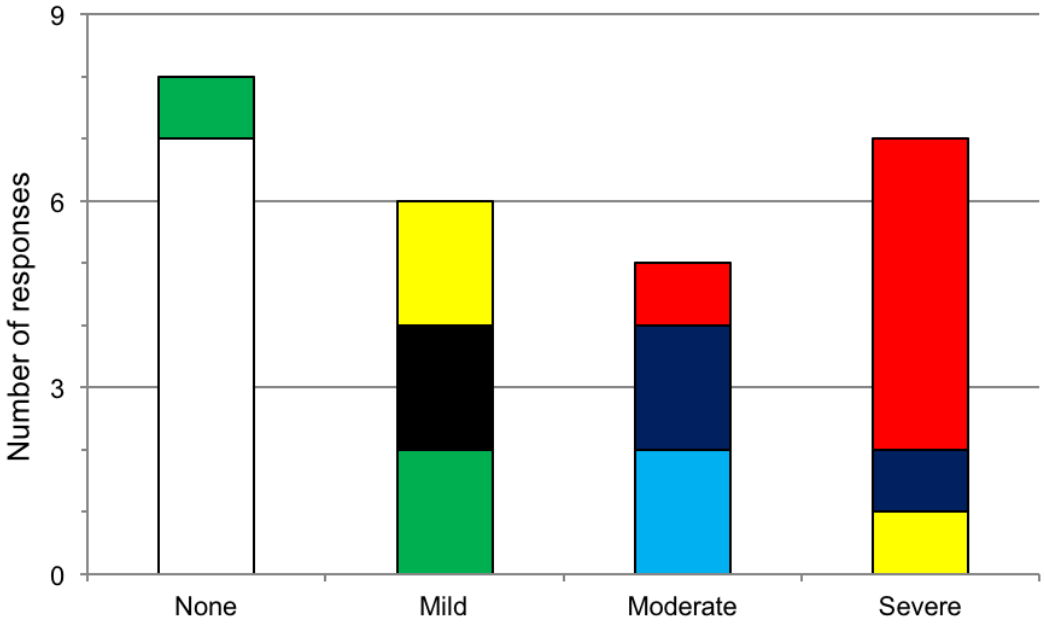


Figure 4.8 Use of colour to indicate pain severity

4.7.5.2. Intensity of self-rated pain on the PPQ.

Figure 4.9 shows the severity of present and worst pain reported by patients and their parent on the PPQ. White was appropriated to signify no pain and red to signify severe pain. Graduations of pink indicated increments on the scale as shown on the right hand side. This shows that approximately half the patients did not have severe pain and that very high levels of pain were uncommon.

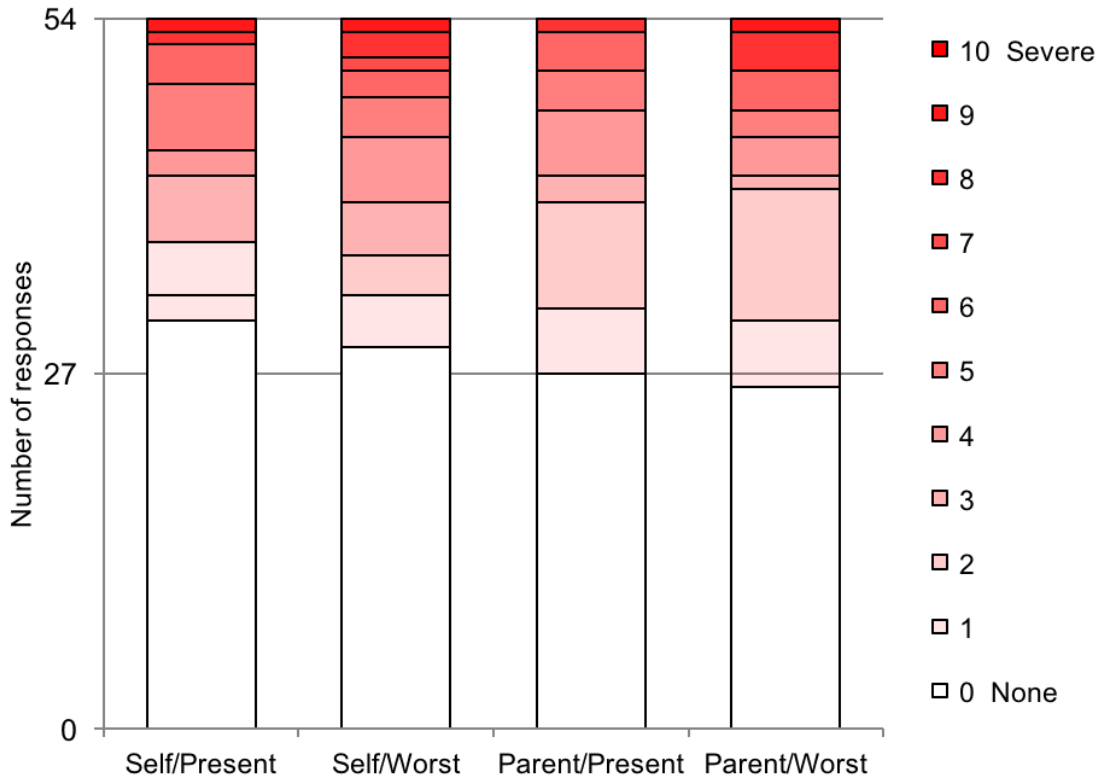


Figure 4.9 Report of pain intensity on the PPQ-VAS

There are highly significant positive coefficients between child and parent pain ratings (self /child report $p<0.001$, parent $p<0.001$, Spearman's correlations). Ratings of worst pain tended to be marginally higher than present pain, particularly for parent report but the results were not statistically significant (self /child report $p=0.464$; parent report $p=0.079$, Wilcoxon Test). The results are displayed as bubble charts in figures 4.10 and 4.11.

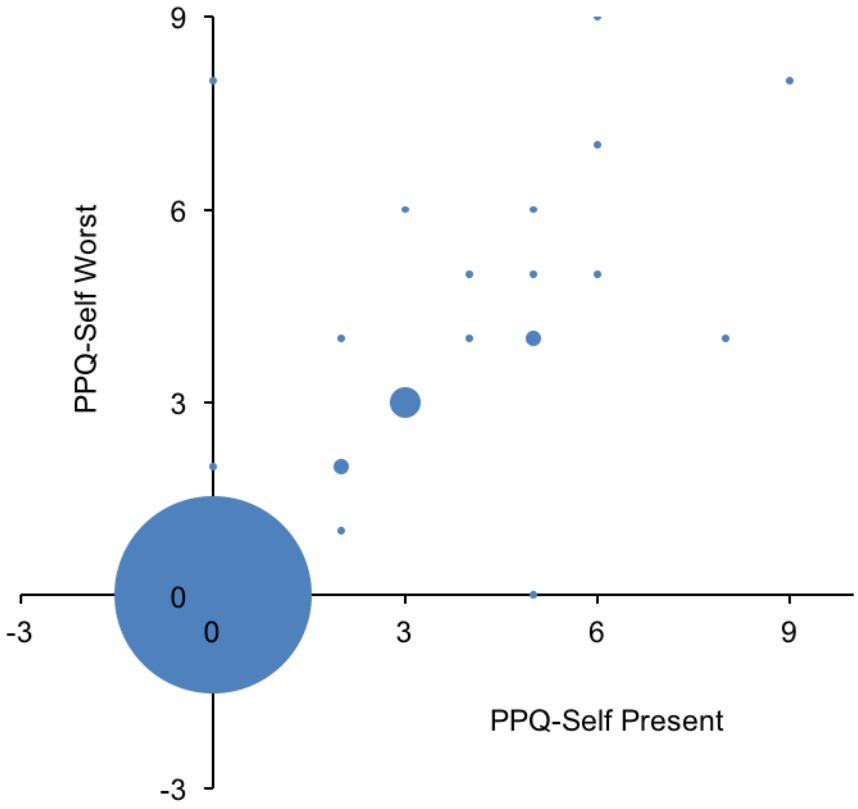


Figure 4.10 Bubble charts of Present and Worst Pain on PPQ child rating.
The bubble diameter indicates the number of responses; therefore, the majority of children rated their present and worst pain as low, with a small number of children reporting very severe pain.

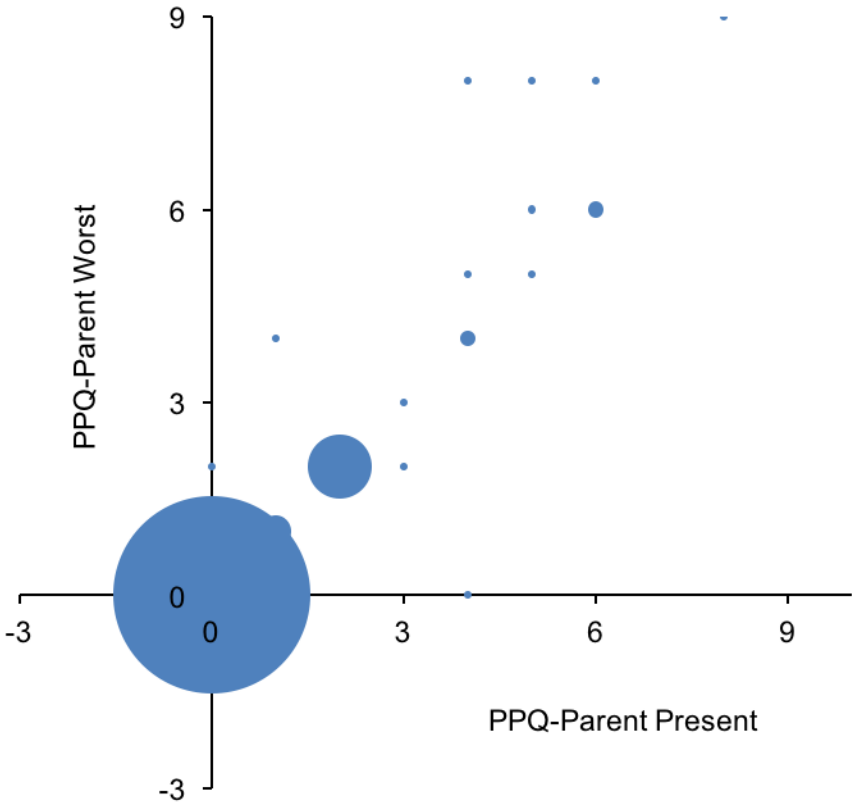


Figure 4.11 Bubble charts of Present and Worst Pain on PPQ Parent rating.
The bubble diameter indicates the number of responses; therefore, the majority of parents rated their child's present and worst pain as low, with a small number reporting very severe pain.

Averaged values of pain report on the PPQ were calculated from present and worst pain for both children and parent reports and indicated a strong and statistically significant relationship between child and parent ratings of pain ($p<0.001$, Spearman's correlation). Ratings of pain severity did not differ significantly between child and parent reports ($p=0.757$, Wilcoxon Test).

4.7.5.3. PPQ ratings and Gender

Consistent with the results above, median pain ratings were generally low for both female and male children on both child and parent report. Pain intensity ratings tended to be higher for females than males, but differences were not

statistically significant (child rating p=0.61, parent rating p=0.18, Mann-Whitney U Test). The results are provided in appendix 4.2.

4.7.5.4. PPQ ratings and LSL type

Median ratings of pain were low for all 3 lipoma types. Both child and parent ratings tended to be higher for transitional lipomas but this finding was not statistically significant (child-report p=0.25; parent-report, p=0.18, Kruskal-Wallis Test). The results are provided in appendix 4.3.

Figure 4.12 shows the median PPQ pain ratings by LSL type.

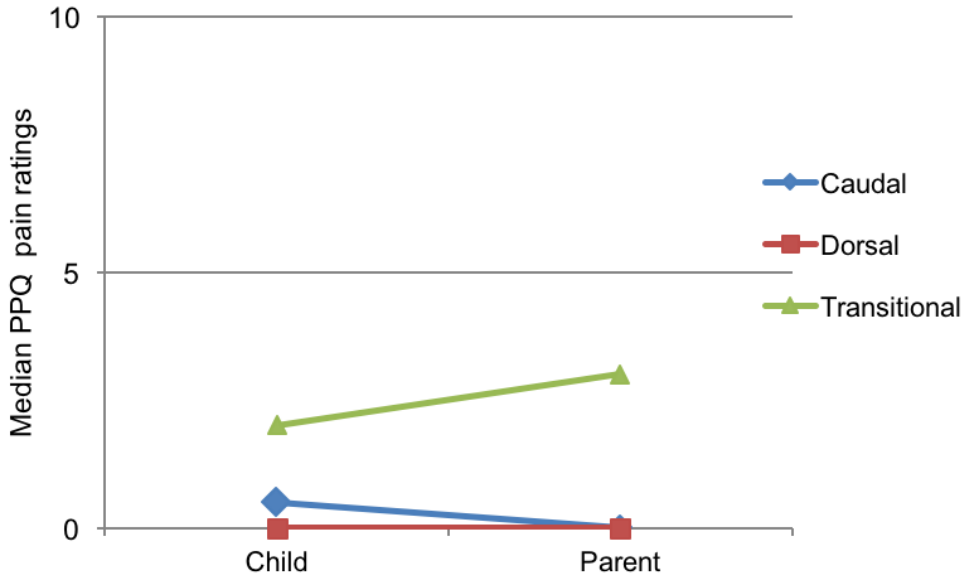


Figure 4.12 Median PPQ pain ratings by LSL type

The figure shows the trend for parents of children with transitional lipomas to rate their child's pain as higher than the child, and higher than the caudal and dorsal groups.

4.7.5.5. PPQ ratings and Syrinx

Median pain ratings tended to be low for patients regardless of the presence of a syrinx, but both children and parent ratings tended to be higher for those

without and approached statistical significance for parent ratings (p=0.07). The results are shown in appendix table 4.4.

4.7.5.6. PPQ and NEM ratings

Spearman's Rank Order Test was used to evaluate correlations between ratings on NEM clinical scales and averaged child and parent report on the PPQ. The correlation coefficients are presented in table 4.5

Table 4.4 Correlations between NEM and PPQ ratings

With the exception of bowel function, the correlation coefficients are negative. This indicates that NEM levels of functioning tended to decrease as pain increased.

Correlations between NEM and PPQ-VAS ratings				
	Child (n=54)		Parent (n=54)	
	r_s	p	r_s	p
NEM Motor	-0.19	0.16	-0.14	0.31
NEM Sensory	-0.38	0.005	-0.31	0.02
NEM Urology	-0.14	0.32	-0.17	0.22
NEM Bowels	0.11	0.42	0.06	0.67

P ≤ 0.05

P ≤ 0.01

However, the only statistically significant relationships were between the NEM Sensory Scale and both child- and parent-reported pain.

As expected, there was a significant negative correlation between impairment on the sensory scale and pain (i.e. higher pain report, which is an important component of this subscale, was associated with lower function). Increased pain tended to also be associated with impairments of motor and urologic function (i.e. negative correlation), which may be suggestive of more severe or more symptomatic disease in patients with pain, but these correlations did not reach statistical significance.

4.7.5.7. PPQ ratings: Summary.

There was also a strong correlation between child and parent ratings of pain intensity, suggesting a degree of validity in report of pain on the PPQ.

In addition, there was significant concordance between ratings of present and worst pain by both children and parent reports. This may suggest that substantial fluctuations in pain over a period of a week were not common, or that both parent and children tended to remember or report levels of pain that were similar to current experience.

Overall, approximately half of patients reported a degree of pain. This was concordant with reports in the categorical measure of pain reported above. Reporting of severe pain was comparatively rare.

4.8. Physical Activity

Patient activity levels were measured using the developmentally appropriate form of either the Physical Activity Questionnaire for Children or the Physical Activity Questionnaire for Adolescents (PAQ-C or PAQ-A; Kowalski et al., 2004). A total of 34 children (63% of the entire sample) completed the questionnaires.

Assessment of the data for normality indicated that the responses on the PAQ-C were normally distributed ($p = 0.02$ Kolmogorov-Smirnov).

However, PAC-A responses were negatively skewed and as a consequence their combined distribution was also not normal; non parametric analysis was therefore used.

4.8.1. Comparison of PAQ to normative data

Patient PAQ scores were compared to English normative data reported by Voss et al (2013). For the purposes of non-parametric analyses, the mean was assumed to approximate to the normative median.

4.8.2. PAQ and Gender

Reported activity levels were higher for females than males but this difference was not statistically significant (p=0.38, Mann-Whitney U Test).

4.8.3. PAQ and LSL type

Although the Kruskal-Wallis Test showed that LSL type did not have a statistically significant effect on activity (p=0.15), there was a trend for the transitional group to report lower levels of activity. The results are presented as a bubble chart in figure 4.13

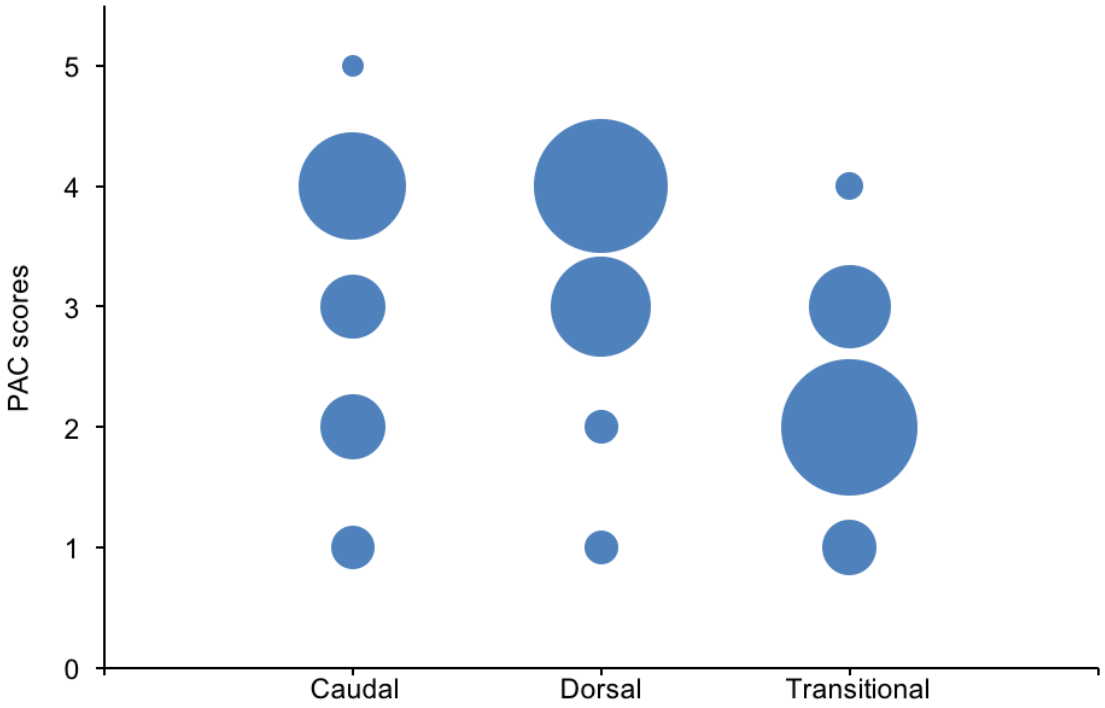


Figure 4.13 Bubble chart of PAQ scores by lipoma type

Bubble diameter indicates the proportion of responses within each lipoma type; the transitional group tended to report lower levels of activity (i.e.: lower scores on the y axis) than the two other groups.

4.8.4. PAQ and the presence of a syrinx

Reported activity levels were higher in patients without a syrinx than those with a syrinx, although this was not of statistical significance (p=0.46, Mann- Whitney U Test).

4.8.5. PAQ and NEM

There was a statistically significant correlation between NEM sensory rating and PAQ ratings (p=0.002, Spearman's rank-order correlation). The correlation with Motor rating only marginally failed to reach statistical significance (p=0.06). The results are displayed in table 4.6.

Table 4.5 Correlations between PAQ and NEM ratings

Correlations between PAQ and NEM ratings								
	NEM Motor		NEM Sensory		NEM Urology		NEM Bowels	
	r_s	p	r_s	p	r_s	p	r_s	p
PAQ	0.331	0.06	0.505	0.002	0.020	0.91	-0.09	0.62

P ≤ 0.05

P ≤ 0.01

4.8.6. PAQ and Pain

The correlations between physical activity measured by the PAQ, and pain as measured by child and adult report on the PPQ, were not statistically significant (child p=0.182; parent p=0.452, Spearman's rank-order correlation). Although high levels of pain would be expected to adversely influence activity levels, within the current sample, the majority had low pain scores and activity levels within the normal range.

4.8.7. PAQ scores: Summary

Analysis of the PAQ data showed that for the group as a whole, activity levels were within the normal range.

Activity levels were not found to be significantly related to most other variables, including gender, LSL type, the presence of a syrinx, or child/parent report of pain.

Of the NEM ratings, the PAQ was statistically significantly and positively related to Sensory function (and marginally to Motor function), but not Urology or Bowel function.

4.9. Discussion

The following section provides a discussion of the clinical results and are aligned to two objectives of the thesis:

1. Objectively assessing a group of children with LSL using standardised methods of clinical assessment. In an attempt to provide a standardised method of clinical assessment and documentation, the advantages and limitations of these two assessment charts are discussed in this chapter.
2. To provide a preliminary analysis in identifying if there is a relationship between LSL type and clinical outcomes, specifically neuro-orthopaedic symptoms (including neurology, pain and orthopaedic symptoms) and sphincter function. In addition, to providing a more thorough understanding of this rare condition, these findings may assist in the provision of a prognosis and allow selective treatment policy.

This is the first study to examine these two points and highlights gaps in the literature which need to be addressed in order to understand and assess more fully, children with this rare anomaly. Equipped with such information, clinicians can compare longitudinal assessment of individual patients and compare their outcomes with international and national colleagues with the aim of improving outcomes for children with LSL.

The 54 children in the current study were selected at random and thus their age and LSL type were likely to be similar to the remaining population of children with LSL at the research site; random selection also reduced the risk of

selection bias. Differences in participants in the current study to those included in other studies (Pierre-Kahn et al., 1997, Kulkarni et al., 2004a, Pang et al., 2010) is that firstly, the current study involves children with LSL, rather than a mixed group of children and adults with LSL; secondly that the current study involves children with LSL, whereas many publications discuss children with a broad spectrum of spinal dysraphism, including split cord malformation and thickened filum terminale; lastly, as most publications are written by neurosurgeons, the majority of data has been related to surgical technique and outcomes, with minimal attention given to standardisation of assessment, comparing lipoma type with outcomes, or providing an objective assessment of surgical outcomes versus the natural history of the disease.

Kulkarni et al (Kulkarni et al., 2004a) compared the clinical outcomes of 53 children with lipomas of the conus managed conservatively with those who had undergone surgery, and found there was no significant difference in outcomes between the two groups. As a result, the children in the current study were recruited regardless of whether they had undergone surgery or not, and one of the study aims was to objectively assess the children, regardless of their management strategy.

The clinical assessment methods in the current study were discussed in Chapter 3 and results documented using the Necker-Enfants Malades Hospital chart (NEM chart, chapter 3, Table 3.1) and the Clinician's observation chart (chapter 3, Table 3.2).

4.9.1. Gender

LSL occurs predominantly in girls with a female: male (F: M) ratio of 2:1 and the results from the current study concur with this finding: females were in the majority in each of the 3 LSL groups and that more specifically, females outnumbered males in the dorsal group and this was of statistical significance. Although this finding provides more understanding regarding the disease, the numbers in the study group were small and as such, should be interpreted with caution.

Gender did not appear to exert significant effect on categorical clinical outcomes in the study group, this is in contrast to the study by Wykes et al (Wykes et al., 2012) who identified in their retrospective study of asymptomatic children, that amongst other factors, being female was a poor prognostic feature. The current research cohort included both asymptomatic and symptomatic children whereas the children in the study by Wykes et al involved children who were all asymptomatic. This raises the question that perhaps females who are asymptomatic are at a higher risk of deteriorating than asymptomatic males, and provides a question for future research in terms of potential prognosis for children with asymptomatic LSL.

There was no statistical significance found in the research group between gender, age differences and LSL type. However, recruited children were aged between 5 and 18 years old (this age was selected to increase the number of validated questionnaires that could be completed) and this age selection may have introduced an element of bias.

4.9.1.1. The use of the NEM chart and clinician's chart in assessing gender

There were no ratings on either chart for assessing the effect of gender on clinical outcomes.

4.9.1.2. Summary of gender and clinical outcomes

Females were in the majority within the current study and were in the majority of each lipoma group. However, gender did not appear to exert significant effect on categorical clinical outcomes in the study group.

4.9.2. Categorical clinical outcomes

4.9.2.1. Urology

The current study identified that 68.5% of the total sample had abnormal urological function, of which 44.4% of the total number of children were using

CIC and 24.1% of the whole sample had deteriorating function. Abnormal urology occurred more frequently in the transitional group and least in the dorsal group; deterioration was not associated with a specific lipoma type. Transitional lipomas, when correlated with other factors (age and the presence of a syrinx), have been associated with deterioration in urology (Pang et al., 2010, Wykes et al., 2012) but the association between LSL type and urological abnormalities without confounding factors, has not previously been examined. There were no clinical variables associated with urological abnormality or deterioration in the study group. There was no female prevalence associated with urological abnormalities in the current study group although the literature suggest otherwise: Dorward et al (Dorward et al., 2002) suggests there is a female prevalence, possibly because the conus as identified by MRI scan, lies within the sacral canal more frequently in females; Wykes et al (Wykes et al., 2012) found the prevalence of deterioration in urology higher in females than males and suggested that perhaps sphincter innervation in females may be more susceptible to tethering than males. Further research with larger study groups may lead to a more thorough understanding regarding the pathophysiology of female urology and identify whether a higher prevalence of females to males is a common finding in urological abnormalities in children with LSL.

The existence / development of an “unsafe” (deteriorating) neurogenic bladder remains one of the main indications for neurosurgery and as a consequence, regular, standardised urodynamic investigations are required (Wu et al., 1998). Of the 54 papers in the systematic review, 50 assess bladder function, which emphasises the importance attributed to the assessment and management of bladder function in children with LSL. The literature suggests that urological abnormalities are common in the majority of symptomatic children with spinal lipomas (Kulkarni et al., 2004a, Pang et al., 2010, Pierre-Kahn et al., 1997, Wykes et al., 2012); this finding is consistent with results from the current study, with two thirds of children having urological dysfunction, rendering urological abnormalities the most common abnormal clinical variable identified in the current study. Abnormal urological symptoms included incontinence, stress incontinence, urgency, infections and the requirement for Clean Intermittent Catheterisation (CIC) and concur with findings in many publications describing

spinal lipomas (Maher et al., 2009, Pang et al., 2010, Pierre-Kahn et al., 1997, Kulkarni et al., 2004a). Just under a quarter of the current study group had deteriorating urology at the time of assessment as identified in Table 3.1 Clinician's observation and data collection chart.

Several authors (Kanev and Bierbrauer, 1995, Wu et al., 1998, Wykes et al.) suggest children with more complex spinal lipomas (i.e. the transitional group) are more likely to have abnormal urological function. This finding can be correlated with the pathophysiology of transitional lipomas that involve the conus and consequently the afferent and efferent pathways controlling sphincter function are affected. The results from the current study concur with findings from other publications and suggest the transitional group are at an increased risk of urological abnormalities, deterioration and the requirement for CIC, and the dorsal group the least at risk. The transitional group also rated lower (i.e. worse) on the NEM score for urology, although there was no statistically significant difference between the dorsal and caudal group in the NEM scores.

All children with LSL should have urological evaluation regardless of MRI scan findings/ lipoma type, and stratified according to potential risk. This would ensure mechanisms are put in place in a timely manner to address any clinical changes, but most importantly to identify urological changes which may herald the onset of tethered cord (Maher et al., 2009). Urological evaluation techniques vary across institutions between invasive and non-invasive techniques to investigate detrusor instability and dyssynergic voiding; whatever investigation is utilised, it is important to recognise the normal parameters of the infant bladder and distinguish between the infant's physiologic detrusor sphincter dyscoordination in the pre- continent child, and detrusor sphincter dyssynergy (neurogenic bladder) (Guerra et al., 2014).

The aim of surgery for resection of LSL and untethering of the spinal cord is to improve the child's neurology and pain. Most authors recognise that improvements in urology are less likely to occur following surgical resection and stabilisation may be the best achievable outcome with regards to urological function, thus highlighting the argument in favour of prophylactic untethering of

the spinal cord and potentially preserving existing urological function (La Marca et al., 1997, Wykes et al.).

4.9.2.1.1. The use of the NEM chart and clinician's chart in assessing urology

As described in the methodology chapter, the NEM chart has 5 columns for assessment of urology but does not allow for subtle deterioration, such as an increase in the number of urinary tract infections or an increase in the frequency of incontinence. Recognising the difference between a stable urological deficit and deterioration is an essential assessment in a child with LSL and as such, should be included in all assessment charts for this group of children. The lack of provision for subtle changes on the NEM chart is thus a limitation of its usage.

The clinician's observation chart was identified as providing essential information regarding urological status, but did not provide sufficient detail regarding urological status, for example the use of Oxybutin suggesting the presence of detrusor-sphincter dyssynergy.

4.9.2.1.2. Summary of urology and clinical implications.

Although the current study results do not all reach statistical significance, the trend is towards children with transitional lipomas being more at risk of abnormal and deteriorating urology and the requirement for CIC, than the caudal and dorsal group, the dorsal group having the most favourable outcome. The results suggest the need for more standardised and predictive urodynamic assessments for all children with LSL and more regular assessment for the high risk transitional group. These findings present implications for financial resources, in addition to implications for the individual child.

4.9.2.2. Neurology

Of the children in the current study, 27% of the cohort had abnormal neurological abnormalities, 18.5% had abnormal stable neurology and 9.3% had deteriorating neurology.

Although deteriorating urology is recognised as the primary clinical red flag in identifying tethered cord in children with LSL, neurology and motor function also form an essential part of regular assessment. The red flag system was established to assist in identifying the potential presence of a serious condition, which could lead to irreversible morbidity if untreated / treated inappropriately and is used across health care professionals in hospital and the community (Welch, 2011).

Neurological deterioration often occurs with age and has been attributed to increased stretch on the spinal cord with axial growth spurts (Cochrane, 2008, Kanev and Bierbrauer, 1995). Most authors recognise the importance of surveillance, with 44 of the 54 papers in the systematic review documenting neurology as part of their assessment of children with spinal lipoma. However, there is no consistency identified in the literature with regard to what assessment tools might provide optimal assessment and documentation (May et al., 2013).

The majority of children in the current study group had normal neurology. In keeping with the complex anatomy of transitional lipomas a higher incidence of abnormal neurology was identified in the transitional group and least in the caudal group. The transitional group scored lowest (i.e. worst) for the motor score and the caudal group scored the highest, although this did not reach statistical significance.

Koyanagi (Koyanagi et al., 1997b) identified that age, the size and type of LSL (specifically transitional) was associated with a higher prevalence of neurological abnormality and deterioration. The association between LSL type and neurological abnormalities as an independent outcome measure without

confounding factors has not previously been examined. When these factors were examined in the current study, the findings showed that abnormal or deteriorating neurology was more common in children with a syrinx than those without and that the difference was statistically significant. There were no clinical variables associated with neurological abnormality or deterioration in the study group, and gender was not associated. Correlation with the motor rating of the NEM score with the PAQ (Physical Activity Score) only marginally failed to reach statistical significance (p=0.06), but suggests that abnormal neurology may be associated with reduced physical activity.

Neurology cannot be assessed in isolation and a combination of neurological (including pain), orthopaedic and urological symptoms is collectively known as neuro-orthopaedic syndrome (tethered cord).

4.9.2.2.1. The use of the NEM chart and clinician's chart in assessing neurology

Neuro orthopaedic assessment on the NEM scale is entitled "motor" scoring and provides a gross assessment of the level of ambulation, ranging from a wheelchair user through to an individual with normal mobility. The scale does not allow for deterioration in neurology or mobility, neither does it provide assessment of muscle power. These symptoms when combined with changes in pain and / or urological deterioration may indicate spinal cord tethering and omission in including these symptoms is recognised as a limitation of the motor section of the NEM chart.

The clinician's observations chart has similar limitations and provides minimal data regarding deterioration in neurology / mobility status.

4.9.2.2.2. Summary of neurology and clinical outcomes.

Although the majority of children in the current study group had normal neurology, an increased incidence of abnormal neurology was identified in the transitional group (50% of those with abnormal neurology) and least in the

caudal group (10%). There was also a higher proportion of neurological deterioration in children with transitional lipomas (15%) and this deterioration was identified as highest in children who also had a syrinx. The results suggest the need for more regular neurological assessments and surveillance in children with transitional lipomas and a syrinx, than those children in the caudal and dorsal group.

4.9.2.3. The presence of a syrinx

The presence of a syrinx was identified in 29.6% of the study group and although not of statistical significance was most commonly found in children with transitional lipomas and was least common in the dorsal group (p=0.19); these findings are consistent with the literature (Lazareff, 2011, Pang et al., 2010).

The presence of a syrinx was more common in males than females, although again, this finding was not statistically significant (p=0.756). There was an increased risk of abnormal neurology in children with a syrinx, than those without a syrinx (p=0.04), but there were no other statistically significant findings between the presence of a syrinx and clinical categorical outcomes. There was however a trend for children with a syrinx to have increased pain: analysing categorical pain ratings showed an increase in pain with the presence of a syrinx in over half the study group. Reduced activity levels were identified using the PAQ activity scale and the presence of a syrinx was associated with a lower motor score on the NEM rating; an increase in pain and abnormal neurology in association with a syrinx has been identified in the literature (Pang et al., 2010, Wykes et al., 2012).

Difficulties exist in separating clinical symptoms to the presence of a syrinx alone in children with spinal dysraphism (Koyanagi et al., 1997b) and the association between LSL type and the presence of a syrinx without confounding factors, has not been previously examined. Some authors suggest that the presence of a syrinx, irrespective of the type of LSL, is a poor prognostic feature in terms of clinical deterioration (Iskandar et al., 2001, Wykes et al., 2012,

Xenos et al., 2000a) and can be an indication for surgery (Xenos et al., 2000a). However, Pang et al (Pang et al., 2010) although not specifically discounting the presence of a syrinx as an important prognostic factor, states that good surgical resection of a LSL in a young child, is the one most important prognostic feature.

Wykes et al (Wykes et al., 2012) suggest the presence of a transitional LSL in association with a syrinx, in a young child of female gender is associated with an increased risk of clinical deterioration. The current study suggests that a child with a transitional lipoma and the presence of a syrinx, has a higher risk of abnormal neurology, increased pain and reduced activity levels. Longitudinal studies with large study numbers are required to more fully understand the relationship between the presence of a syrinx and clinical outcomes in children with LSL.

4.9.2.3.1. The use of the NEM chart and clinician's charts in assessing the presence of a syrinx

There were no ratings on either chart for assessing the presence or clinical relevance of a syrinx. However, the numerical ratings on the NEM chart did provide evidence of reduced motor and sensory scores in children with a syrinx in the study group.

4.9.2.3.2. Summary of the presence of a syrinx and clinical outcomes.

A syrinx was found to occur most frequently in the transitional group with a trend towards reduced motor scores, reduced physical activity and increased pain identified in children with a syrinx.

The presence of a large lipoma could have the potential to cause compression of the spinal cord and obstruction of CSF pathways with resulting formation of a syrinx, but the precise aetiology of a syrinx in relation to LSL is not understood and it may be more related to the presence of a tethered cord, than to the lipoma itself (Lazareff, 2011).

The potential prognostic implications of a syrinx in the context of LSL, including the size and location of the syrinx, needs to be prospectively studied.

4.9.2.4. Pain

"Children's pain matters- for the child, for the family and for society" (International Association for the Study of Pain, 1995).

In the current study, pain was reported by approximately half the sample in the categorical pain ratings and was present across all three LSL group, with 21% reporting pain only in the legs and 7% reporting pain in both the back and legs; there were no reports of back pain without leg pain, and no association between pain location and LSL type. Median ratings of pain were low for all three LSL types, however, child and parent ratings tended to be higher for transitional LSL group, although this was not statistically significant. There were no statistically significant differences in reported pain by gender (although there was a trend for female participants to report higher levels of pain than males), by LSL type, or by the presence of a syrinx. An increase in pain was however associated with a decrease in physical activity.

There were no publications identified in the systematic review correlating the presence of pain with other clinical variables in children with LSL, however new onset pain is an important finding in children with LSL as it can herald the presence of tethered cord syndrome and prompt assessment of urology and neurology (Pang et al., 2010). The systematic review identified that 26 of the 54 papers documented pain as part of their assessment of children with LSL, but supplied minimal details of associated factors or pain location or severity.

Averaged pain scores from the Paediatric Pain Questionnaire (PPQ-Self and PPQ–Parent) showed a higher incidence of pain in females, although this was not statistically significant; the categorical data analysis however did not show this prevalence. The presence of pain was identified as higher in females than males in a systematic review examining chronic and recurrent pain in children and adolescents (King et al., 2011) although the authors found that

demographic and psychosocial factors could also be associated with an increased prevalence of pain. Whilst the review by King et al suggested a higher level of pain perception in females with females reporting multiple pain symptoms and higher overall pain scores than males, a study by Blanckenburg et al (Blankenburg et al., 2011) of 173 healthy children, found no gender effects in young children with pain. The authors found that this gender effect changed during adolescence and puberty, when females become more sensitive to sensory changes, in particular to thermal perception thresholds. The effect of age in association with pain and gender was not examined in the current study group due to the small participant numbers, but further research involving larger numbers might yield more detailed results that could further our understanding of pain in this group of children.

In addition, the literature suggests that pain in sick children although frequently reported is often undertreated (Clancy et al., 2005), can lead to functional deficits, reduced participation in physical activity (Gauntlett-Gilbert and Eccleston, 2007) and can have a negative impact on the child's HRQL (Wood et al., 2009a). Chronic pain in childhood may predispose to pain and disability in adulthood due to central sensitisation and therefore early intervention is warranted {Walker, 2012 #2538.

4.9.2.4.1. Pain location

The occurrence of, or increase in back and /or leg pain on exertion in children with LSL is recognised as one of the important factors in heralding the occurrence of tethered cord and in particular, of retethered cord {Pierre-Kahn, 1997 #2107}. Analysis of pain location in the current study identified pain occurred most commonly in the legs, but also occurred in the legs and back together for some patients with caudal and transitional LSL. No child in the current study reported back pain in isolation and this new finding may be of benefit to clinicians when differentiating between children with chronic back pain, and those presenting with a tethered cord.

4.9.2.4.2. Pain scoring

Asymmetrical cord tethering can result in musculoskeletal pain secondary to muscle and joint deformities and musculoskeletal pain is often exacerbated by physical activity with potential avoidance of that activity (International Association for the Study of Pain, 2010). The correlation between physical activity measured by the PAQ and pain measured by the Pediatric Pain score (PPQ) in the study group was not statistically significant, yet there was a statistically significant correlation found between the sensory function on the NEM score (and marginally to the motor score) and physical activity. The reasons for these differences are unclear, but may relate to the fact that the PPQ VAS was scored by the child and parent and the NEM by the clinician. The literature suggests that clinicians often rate the presence and degree of pain in children lower than the child / parent rating and Da Silva (Mathews, 2011) suggests that despite the importance of pain management in children, clinicians may focus more on the source of pain rather than pain assessment, severity and management. In a study of pain in children with cerebral palsy, discrepancies between child / parent pain rating and pain ratings undertaken by clinicians were identified, with 17% of the children stating they had pain, yet none of the clinicians rated these children as having pain (Penner et al., 2013). The current study found that the child / parent rating of pain with activity was lower than the clinician's rating using the NEM scale, and this discrepancy highlights the difficulty is using different methods of assessing the same variables.

In a study of children with spinal dysraphism (comprising myelomeningocele, lipomyelomeningocele and lipomyelocele) with chronic pain, parents were reliable at reporting only their child's severest pain, with parents of younger children in particular underestimating pain intensity (Clancy et al., 2005). This was not the case in the current study where a highly significant concordance was found between ratings of present and worst pain by both children and parents. This suggested that substantial fluctuations in pain over a period of a week were not common. In addition, high levels of reported pain were comparatively rare.

The strong association between child and parent ratings in the current study suggests a degree of validity in report of pain on the PPQ in contrast to the NEM scale, the latter, which has not been validated. The PPQ chart was identified as a validated, useful tool for assessing the study group and provides concordant child and parent reports.

4.9.2.4.3. The use of body maps and colour

Colour to depict pain severity on the body maps was used by 33.3% of children in the current study and although there were some trends in colour choice, red was the colour most used to depict severe (and moderate) pain. These findings are consistent with a study by Varni et al (Varni et al., 2005) who identified red as the colour most used by children to depict severe pain. Figure 4.14 is displayed below rather than in the results section, to allow the reader to visually understand the use of a body map and the use of colour as depicted by a 6 year old girl in the study group.

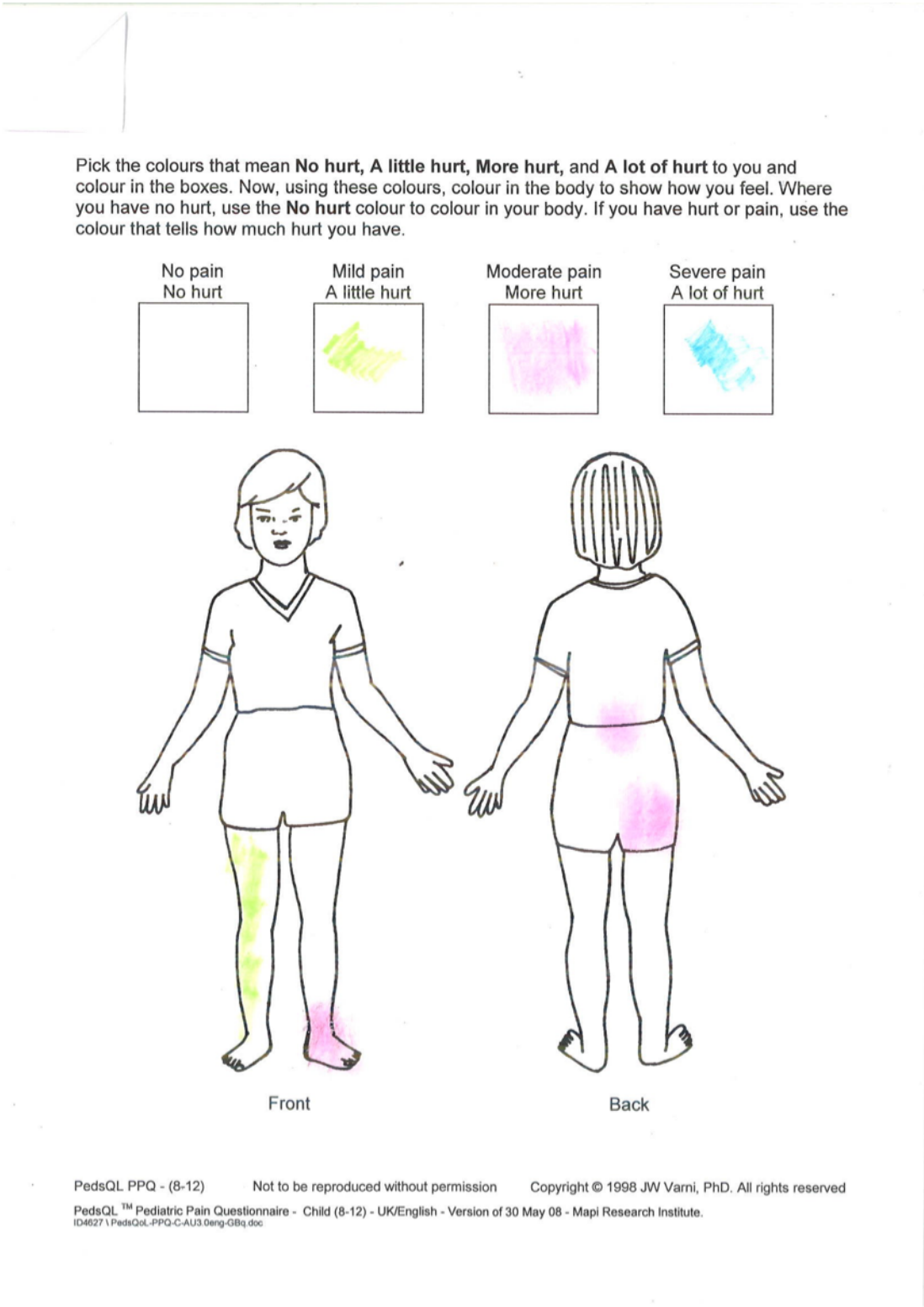


Figure 4.14 Body map

Gragg et al (Gragg et al., 1996) found minimal evidence to suggest that specific interpretation could be made regarding pain using colour alone, but by finding

which colour a child associated with the level of pain, pain interventions could be assessed and consequently acted upon. Minimal research has been undertaken using colour scales to depict pain in children and von Baeyer and Spagrud (von Baeyer and Spagrud, 2007) suggest that colour scales are rarely used in the clinical setting due to the time this takes to administer. There are no publications relating to the use of colour to depict pain in children undergoing neurosurgery; in adults undergoing neurosurgery however, the use of colour to depict pain was found to provide no additional information than the use of black and white drawings (Masferrer et al., 2003). Further research is required to examine the clinical usefulness of coloured pain drawings in children with LSL to ascertain if such methods would assist in determining treatment.

Two of the five year old children in the current study identified their calves as the source of their pain and were wearing calipers; neither child liked their calipers and it was difficult to decipher if marking the area of their calves on the body map represented pain, or irritation at wearing the calipers. As described in the literature (Walco et al., 1992), body maps completed by the child can assist the clinician in identifying specific pain locations the child may not be able to verbalise, or that may not have been previously identified. However, Von Baeyer et al (von Baeyer et al., 2011) suggest young children can find body maps confusing as they may not be able to point to specific painful body parts and may also be embarrassed at being presented with nude body outlines. The authors also suggest that body maps may provide unintended suggestions of age, gender and ethnicity. A systematic review of tools for locating pain in children undertaken in 2014 (Hamill et al., 2014), refers to the continued complexity of using and analysing such tools and the requirement for further research. The use of body maps to depict pain location, and colour to depict pain severity, (with additional descriptors if provided) continues to be an area of research in the assessment and management of children's pain.

Varni et al (Varni et al., 1987) suggests useful additional information can be provided by children's pain descriptors. Children in the current study who described their pain, used the following terminology: "spikey", "severe", "continuous", "ouch", "cramps", "fireworks", "upsetting". This terminology

suggests the need to address and manage pain in this group of children more efficiently.

Analysis of the body map section, the use of colour in the PPQ questionnaire and the pain descriptors provide useful information on pain assessment tools in the study group but further research is required to fully analyse and utilise these tools.

4.9.2.4.4. The use of the NEM chart and clinician's chart in assessing pain

The NEM chart consists of five sections for sensory assessment with section one entitled "skin ulceration and amputation". Amputation is an unlikely scenario in children with LSL and as such, renders the tool less appropriate for assessing children with LSL. Furthermore, the NEM scale does not provide a description of the location or severity of pain and this omission presents an additional limitation of the scale.

The clinician's observation chart provides the clinician with the ability to assess for pain in the back / legs and whether pain occurs with physical activity, but it does not provide the ability to assess and score pain severity.

4.9.2.4.5. Summary of pain and clinical outcomes

There were no statistically significant differences in reported pain by lipoma type, gender or the presence of a syrinx. Pain was however present in over half of the children in the current study and occurred more frequently in the transitional and least in the dorsal group. Pain was not found to occur in the back only, but occurred in the back and / or legs on exertion and this finding should alert the clinician to the child's potential clinical deterioration and prompt further investigations.

Pain intensity has been described as mild if the pain score is less than 4 (out of 10) and severe if 7.5 or above (Hawker et al., 2011). There was concordance in the study group between child and parent pain ratings, with over half the group

reporting pain. The majority reported predominantly mild pain (<4), (PPQ child n=10; PPQ parent n=13); severe pain (> 7.5) was only reported by a small number of participants (n=2: PPQ child and parent score). On the NEM ratings, a lower activity level as rated on the PAQ data was associated with increased pain and reduced motor function, although for the group as a whole, activity levels were within the normal range.

The current study suggests there is a gap in the current management of pain in children with LSL and further research is needed to assess, understand and manage pain more effectively in this group of children.

4.9.2.5. Orthopaedic abnormalities

Orthopaedic abnormalities were identified in 20.4% of children in the current study and occurred most frequently in the transitional group and least in the caudal group. Transitional lipomas are more commonly associated with rotation of the spinal cord with resulting asymmetry, neuromuscular imbalance and progressive deformity as the child grows and this can result in orthopaedic abnormalities including asymmetrical leg length, cavovarus, cavus, equinocavovarus, hip dislocation and progressive scoliosis (La Marca et al., 1997, Pierre-Kahn et al., 1997). In contrast, children with dorsal and caudal lipomas are less likely to have evolving orthopaedic abnormalities, as rotation of the spinal cord is unlikely. There was no association found between orthopaedic abnormalities and any other categorical clinical variable or with gender or pain in the current study.

Congenital skeletal deformities are reported in one third of children with spinal lipoma at initial presentation (Pierre-Kahn et al., 1997) and 38 of 54 papers in the systematic review included orthopaedic assessment as part of their overall assessment criteria. In their study of children with lipomyelomeningocele, Segal et al (Segal et al., 2013) identified 41% of children with orthopaedic abnormalities, but highlighted the paucity of publications reporting orthopaedic abnormalities as an isolated finding.

In contrast to the current study findings, Tubbs et al (Tubbs et al., 2006) identified that a caudal lipoma was more likely to be associated with orthopaedic abnormalities in their study of 25 patients with lipomyelomeningocele, although no potential reason for this finding was provided. No other publications were identified in the systematic review linking orthopaedic abnormalities to a specific LSL type, and the majority of the literature described neuro-orthopaedic syndrome, encompassing a combination of neurology, urology and orthopaedic symptoms.

4.9.2.5.1. The use of the NEM chart and clinician's chart in assessing orthopaedic abnormalities

Neither chart provided depth of detail or allowed for measurement of subtle deterioration in orthopaedic deficits and functioning. As previously described, the "motor" section of the NEM scale combined neurological and orthopaedic symptoms but provided only a gross assessment of the level of ambulation. The clinician's observation chart consists of three sections, - foot deformity, ankle deformity and scoliosis, but there is no provision for the assessment of subtle deterioration.

4.9.2.5.2. Summary of orthopaedic abnormalities and clinical outcomes

Neuro-orthopaedic syndrome (tethered cord) describes the combined deterioration in orthopaedic, neurological and urological function. Orthopaedic function therefore cannot be assessed in isolation but as part of the overall picture.

With the exception of the publication by Segal et al, there were no studies in the systematic review that examined orthopaedic status as an isolated variable, with the majority of studies describing neuro-orthopaedic syndrome (Segal et al., 2013). The current study identified orthopaedic abnormalities occurring more frequently in children with transitional lipomas and this was consistent with the

findings from the study by Segal et al. Orthopaedic abnormalities were associated least in the caudal group in the current study, this has not been previously identified in the literature.

4.9.2.6. Bowel function

The assessment of bowel function is discussed in combination with urological assessment in the majority of publications in the systematic review, with 24 of the 54 papers in the review including bowel function in their assessment of children with LSL. The association between LSL type and abnormality / deterioration in bowel function without confounding factors, was not identified in the systematic review. The current study is the first to examine abnormal bowel function in isolation in children with LSL and 10 children (18.5%) were identified as having abnormal bowel function in the presence of normal urological function. Bowel abnormalities were identified in over half of the children in the current study of which 35.2% had abnormal but stable function and 9.3% had deteriorating function. Abnormal bowel function was more common in the caudal and least common in the dorsal group as identified by the mean rank analysis from the NEM data. The clinical categorical results show a higher trend in deterioration in bowel function for the transitional group than the caudal group, with none of the dorsal group having deteriorating bowel function.

There was no association identified between bowel abnormalities and gender, the presence of a syrinx or any clinical categorical outcomes in the current study.

4.9.2.6.1. The use of the NEM chart and clinician's chart in assessing bowel function

The NEM chart consists of three parameters, - incontinence / stoma, painful constipation or normal bowel function. The clinician's observation chart consists of three sections, - constipation, urgency and incontinence. A limitation of both charts is the omission for documentation of subtle deterioration in bowel function.

4.9.2.6.2. Summary of bowel function and clinical outcomes

Abnormality in bowel function was most common in the caudal and least common in the dorsal group, with deterioration identified in the transitional and caudal group only. There is not an association identified in the literature between deterioration in bowel function and tethered cord in children with LSL and dysfunction in defaecation is given minimal discussion in the systematic review. However, faecal incontinence can have a considerable negative effect an individual's HRQL (Daszkiewicz et al., 2007, Wide et al., 2014) and must be considered in the overall management of children with LSL.

This section has provided the statistical analysis for the clinical variables of children with LSL. The results will be discussed in the following section.

4.10. Discussion

4.10.1. The first aim of the chapter

The first aim of this chapter, to objectively assess a group of children with LSL using standardised methods of clinical assessment and discuss the advantages and limitations of two assessment charts, has been achieved:

All 54 children in the current study have been assessed using consistent, standardised methods of clinical assessment as outlined in the methodology chapter. The results have provided data on which to objectively assess the child's current status and to assess outcomes of interventions including surgery. Urological abnormalities were found to be the most common symptom in the majority of symptomatic children with LSL in the current study and this is consistent with the literature. Although the majority of the children in the current study had normal neurology, variations in neurological assessment methods and documentation in the literature, made comparison between the study group and data from the literature difficult, highlighting the importance of standardised assessment and documentation. The presence of a syrinx and associated increase in pain and reduction in physical activity is highlighted in the current

study and suggests the need for further research to understand the pathogenesis and prognostic significance of a syrinx in the context of LSL. Although pain was reported by approximately half the study group, several different methods of pain assessment were undertaken, resulting in inconsistent results, again highlighting the importance of consistent standardised assessment.

The advantages and disadvantages of using the NEM and the clinician's assessment forms were discussed: The NEM chart was useful in identifying gross changes in function and provided numerical data which could enable statistical analysis, whereas the clinician's observation chart provided the majority of criteria by which to assess the child with LSL. Neither chart however, provided sufficient detail for assessing early, subtle deterioration in clinical findings some of which may be irreversible. Deterioration in symptoms often heralds the presence of tethered cord and the consequent aim of prompt surgery is to stabilise symptoms and prevent further deterioration. Finally, neither the NEM chart or the clinician's chart have been validated or tested for interreliability, thus highlighting the importance of a new, standardised chart that meets the requirements discussed.

Health related quality of life (HRQL) was omitted from both the NEM and clinician's assessment chart, yet chronic disease can affect the child's development as it can change the developmental trajectory, has implications for future mental health and can help identify future health care requirements (Verhoof et al., 2013). An assessment of HRQL should therefore be incorporated into the child's clinical assessment. The HRQL of children with LSL is discussed in chapter 5.

4.10.2. The second aim of the chapter

The second aim of this chapter, to provide a preliminary analysis in identifying if there is a relationship between lipoma type and clinical outcomes, specifically neuro-orthopaedic symptoms (including neurology, pain and orthopaedic symptoms) and sphincter function, has been achieved.

The association between LSL type and individual clinical variables without confounding factors has not previously been examined. The anatomical site of a lipoma correlates with clinical outcomes, with transitional lipomas involving the conus for example, having a higher risk of urological dysfunction. Rotation of the spinal cord and resulting asymmetry is more common in transitional lipomas with a potential increased risk of neuro-orthopaedic abnormalities and deterioration with age as the spinal cord elongates, compared to children with dorsal and caudal lipomas. Overall, there was an increased trend for the transitional group to have a higher risk of abnormal neurology, urology, and orthopaedic abnormalities, higher reported pain, and reduced levels of physical activity. There was a trend for the dorsal group to have the lowest risk of abnormalities in bowel, urology and reported pain, with the caudal group having the lowest risk of abnormal neurology and orthopaedic deficits. These findings assist in the provision of a prognosis and allow selective treatment policy.

4.11. Summary

Chapter 4 discussed the clinical assessment outcomes of children with LSL and demonstrated that clinical parameters are not equally distributed across LSL types. This suggests that children could be risk stratified according to lipoma type, with more intensive initial investigations and selection for potential aggressive early surgery in children with transitional lipomas and the presence of a syrinx.

The NEM chart and the Clinician's assessment chart used in the study did not provide the detail required for the study group and suggests the requirement for a new method of assessment and documentation to enable surveillance, early recognition of deterioration, and provision of an objective assessment of surgical outcomes versus the natural history of the disease in children with LSL. A draft outcome / assessment form for children with LSL will be provided at the end of the thesis.

Additional novel findings were identified in the study: The fact that no children reported back pain in isolation for example, is a novel finding; a further new

finding from the study, identified that abnormalities in bowel function can occur in the presence of normal urological function. Such new knowledge has increased our understanding about LSL and can inform and guide clinicians.

Childhood chronic illness can have a negative effect on the child's Health Related Quality of Life yet this has not been examined in children with LSL. The following chapter presents these findings.

Chapter 5. The Health Related Quality of life of children with lumbosacral lipoma

5.1. Introduction

“Function, Impairment, and Disability are words in which many physicians have little interest”(Chen, 2007).

The focus of the management of patients with chronic health conditions including that of children with lumbosacral lipoma (LSL) is moving away from looking purely at clinical outcomes. A central goal in the provision of medical care is to increase or optimise the patient’s health related quality of life (HRQL) and there is an increasing awareness of the importance of measuring the impact of treatment strategies and outcomes on the individual’s perception of their HRQL.

The aim of this chapter is to describe the HRQL of children with LSL and to explore the association between HRQL and the clinical variables associated with the condition. This association has not been previously examined and consequently was undertaken to explore the burden of LSL on the patient and family. There are a number of HRQL assessment tools. These assessment tools are often unwieldy and may not be appropriate for specific conditions. In order to identify the most important parameters to assess, three HRQL tools were chosen:

- The PedsQL 4.0. Generic core scale (Varni et al., 1999). As described in chapter 3, the PedsQL scale is a generic questionnaire containing 23 items which contribute to 4 sub scores (physical, emotional, social, school) from which a total score can be calculated. Both parent-proxy and child versions of the PedsQL questionnaires for children between 5 and 18 years of age were used in this study.
- The Child Health Questionnaire self (CHQ-CF87) and proxy (CHQ-PF50) (Landgraf, 1999). The CHQ-CF87 is a generic measurement of HRQL for

children between 10 and 18 years of age and contains 87 items which measure the physical, psychological and social well-being of the child. The CHQ-PF50 is a generic proxy measurement completed by parents / carers of children between 4-18 years of age and contains 50 items that contribute to physical and psychosocial scores based on 12 specific domains, and two composite scores.

- The Piers- Harris Children's Self Concept Scale (PH2) (Piers and Herzberg, 2002) was used to assess the children's self-concept / self-esteem. The scale is a validated self-report questionnaire for children aged 7-18 years and is used to measure self-esteem and how a child feels about himself / herself.

This chapter describes in detail the results from these HRQL questionnaires in relation to child and parent burdens. In addition, the results from HRQL questionnaires were correlated with clinical variables to provide data related to specific physical deficits; finally, the associations between lipoma type and specific clinical deficits were investigated in relation to HRQL. Our findings were used to inform the development of a tool which was used within the clinical setting to provide consistent and standardised overall assessment including HRQL, on which to base a management plan.

5.2. Why measure HRQL?

LSL is a chronic disease that has the potential to deteriorate over time; it is well known that HRQL evaluation in the context of chronic disease may assist in providing timely and appropriate physical and psychological management and interventions (Anderson et al., 2015). This was the motivation for studying HRQL in children with LSL.

5.3. The aim

One of the thesis aims is to explore the impact of LSL on the child. This chapter examines the HRQL of the child to determine if lipoma type and specific clinical deficits are related to HRQL

Our findings can then be used to inform the development of a tool which can be used within the clinical setting to provide consistent and standardised overall assessment including HRQL, on which to base a management plan.

5.4. Methodology

The methodology by which the HRQL of children with LSL was assessed is provided in chapter 3.

In keeping with the previous chapters, analysis was undertaken using the NEM scale (Kulkarni et al., 2004a, Pierre-Kahn et al., 1997). As described in the methodology section, the NEM score comprises 4 subscales including motor function (maximum score of 5), sensory (maximum score of 4), urinary (maximum score of 5) and bowel function (maximum score of 3), with an overall maximum score of 17, the higher score depicting higher function. The NEM scale is provided in appendix 4.1.

5.5. Results of Psychosocial outcomes

All 54 children who were clinically assessed and their parents completed the questionnaires in full. Only the statistically significant findings are shown in the main text. For further reference, the findings that were not statically significant are provided in appendices.

5.5.1. PedsQL measure of Quality of Life

HRQL was measured using patient and parent report on the Pediatric Quality of Life Inventory (PedsQL), including a Total score, a score for Physical Functioning and a Psychosocial Health summary score derived from separate measures of Emotional, Social, and School functioning. Proxy ratings are hereafter referred to as parent ratings; self-ratings are hereafter referred to as child ratings.

Assessment of the data for normality indicated that the pattern of responses on all scales was negatively skewed. Distributions for all child report and the

majority of parent report scales violated assumptions of normality (appendix, table 5.1). Consequently, non-parametric analyses were used.

5.5.1.1. Correlations between PedsQL domains for child and parent report

All correlations between PedsQL domains for child and parent report were statistically significant, typically at the $p<0.001$ level (Spearman rank order correlations) and are provided in appendix table 5.2

5.5.1.2. Comparison of LSL PedsQL data with normative data

The PedsQL scores were compared to UK normative data for healthy children reported by Upton et al (Upton et al., 2005) and are provided in appendix 5.3 The author's normative values were reported in terms of means and standard deviations. Their means were assumed to approximate to the normative median for the purposes of non-parametric analyses.

One-Sample Wilcoxon Signed Rank tests were used to evaluate the significance of the differences between LSL children and healthy normative children. Both child and parent ratings of Physical Functioning were significantly lower than the normative values. None of the self-reported psychosocial ratings or the Total score differed significantly from the normative values. However, parent ratings were significantly lower for the Social domain, the psychosocial composite and Total score. Comparison of child ratings and normative values are displayed in figure 5.1 and the statistical analysis results are presented in table 5.1. Comparison of parent ratings and normative values are displayed in figure 5.2 and the statistical analysis results are presented in table 5.2.

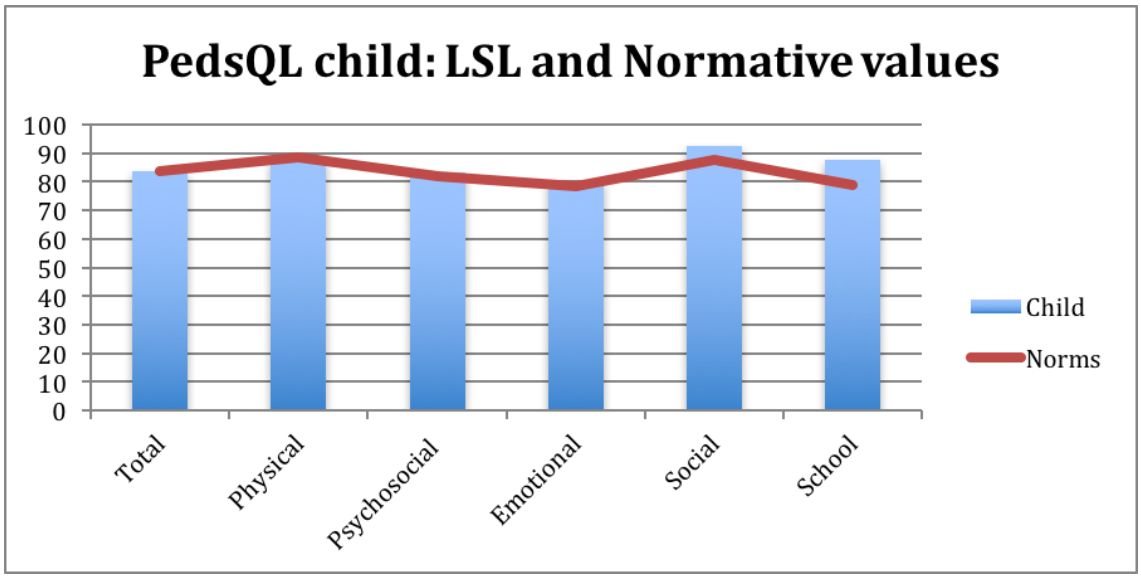


Figure 5.1 PedsQL child: LSL and normative values

The x axis provides the domains of the PedsQL and the Total score; the y axis provides the score (maximum score =100) for each of these domains. The graph demonstrates that the LSL group ratings were similar to normative values.

Table 5.1 Comparison of LSL and normative PedsQL data: Child (Upton et al., 2005)

Physical Functioning was the only domain that was significantly lower than the normative values (norms). IQR: Interquartile range.

		LSL	LSL	Norms	P value
Child	N	Median	IQR	Mean/Median	p
Total	54	83.70	25.82	83.89	0.27
Physical health	54	87.50	25.78	88.51	0.03
Psychosocial health	54	83.33	28.33	81.84	0.55
Emotional	54	80.00	40.00	78.49	0.58
Social	54	92.50	30.00	87.65	0.63
School	54	87.50	35.00	78.87	0.46

p ≤0.01 p≤0.05

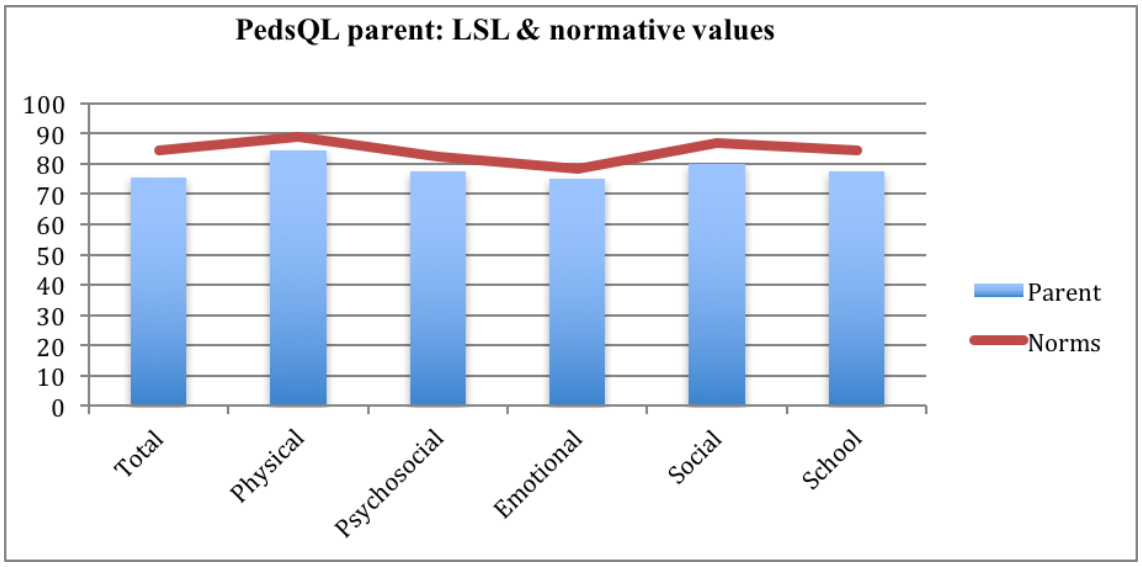


Figure 5.2 PedsQL parent: LSL & normative values

The x axis provides the domains of the PedsQL and the Total score; the y axis provides the score (maximum score = 100) for each of these domains. The graph demonstrates that parent ratings for the LSL group were lower than normative values on all domains.

Table 5.2 Comparison of LSL and normative PedsQL data: Parent (Upton et al., 2005)

All parent ratings were significantly lower than normative values with the exception of the Emotional and School domains.

		LSL	LSL	Norms	P values
Parent	n	Median	IQR	Mean/Median	p
Total	54	75.54	25.65	84.61	0.002
Physical health	54	84.38	38.28	89.06	0.005
Psychosocial health	54	77.50	32.08	82.21	0.02
Emotional	54	75.00	32.50	78.28	0.14
Social	54	80.00	36.25	86.82	0.003
School	54	77.50	35.00	81.52	0.171

p ≤0.01 p≤0.05

Figure 5.3 shows the difference between obtained and normative PedsQL values for self and parent report.

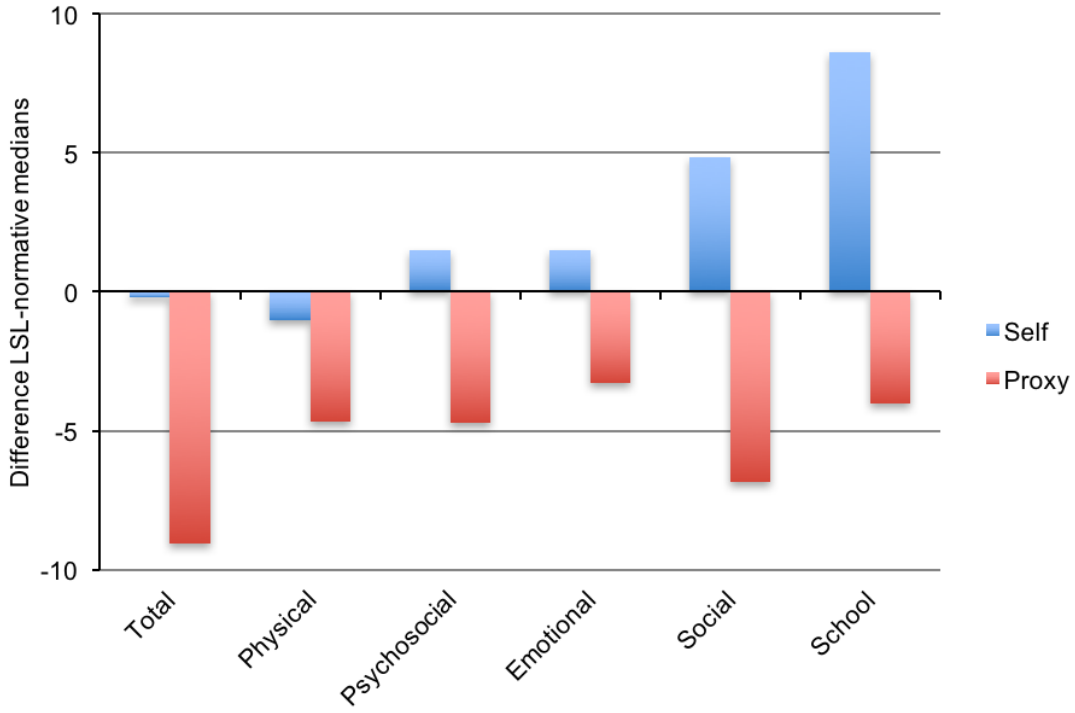


Figure 5.3 Difference between obtained and normative PedsQL values for self and parent report.

The x axis shows the PedsQL domains and the Total score. The y axis shows the variation of child and parent ratings where the normative value is scored as 0. The figure shows that shows that parent report was consistently and often apparently substantially below the normative value. In contrast, the direction of differences for child report was not consistent, including a possible dissociation between lower-than-normative Physical Health but higher on all Psychosocial measures.

Table 5.3 Comparison of child and parent PedsQL report

Wilcoxon Signed Rank Tests indicated that the differences between child and parent ratings were statistically significant for the Total, the Psychosocial composite, and the Social domain.

Comparison of self and parent PedsQL report						
	Total	**Physical**	**Psychosocial**	**Emotional**	**Social**	**School**
z	-2.518	-1.180	-2.585	-1.299	-3.055	-1.295
p	0.01	0.24	0.01	0.162	0.002	0.195

p ≤0.01 p≤0.05

5.5.1.3. PedsQL and child gender

The results (appendix table 5.4) indicated that females tended to self-report higher HRQL than males on all Psychosocial scales and a similar level on the Physical scale. However, only the difference in Emotional quality of life was statistically significant (U= 204.5, p=0.026 Mann- Whitney U test).

Consistent with the analyses of the effect of child versus-parent report for the whole sample, parent ratings tended to be lower than self-report for females on all scales. The same trend was also apparent for males on most scales. Males self-reported significantly lower Emotional HRQL than females, this was in contrast to parent ratings whereby parents reported males as having a higher Emotional HRQL than self-report and similar to females.

5.5.1.4. PedsQL and LSL type

The median PedsQL scores for child (Figure 5.4) and parent (Figure 5.5) ratings for the three LSL types are shown below.

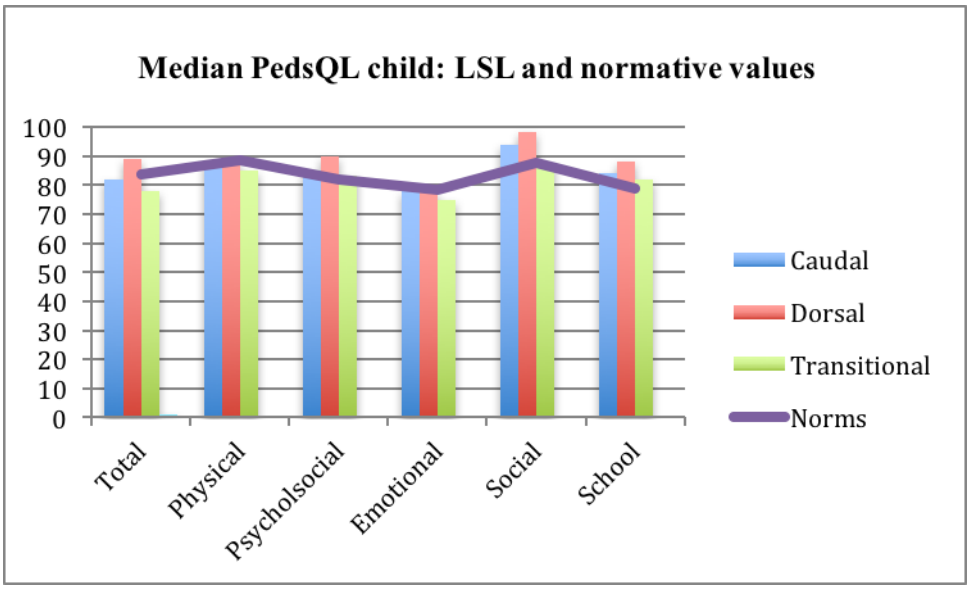


Figure 5.4 PedsQL child, LSL and normative values

The x axis provides the domains of the PedsQL and the Total score; the y axis provides the score (total score = 100) for each of these domains. Child report for the transitional group shows they have the lowest reported HRQL; dorsal groups reported the highest ratings in the majority of domains.

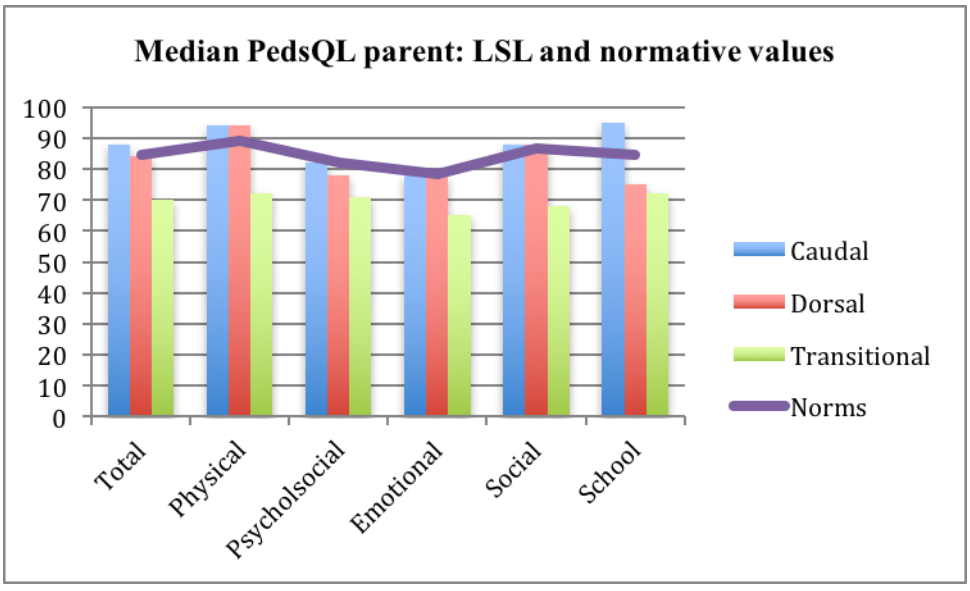


Figure 5.5 PedsQL parent, LSL & normative values

The x axis provides the domains of the PedsQL and the Total score; the y axis provides the scores (maximum score = 100) for each of these domains. Parent report for the transitional LSL group shows this group have the lowest reported HRQL and in contrast to child ratings, parent ratings indicate the caudal group have the highest HRQL in most domains.

The group differences in child report of HRQL were not statistically significant across lipoma groups on any of the subscales (Kruskal-Wallis Test of group differences). The results are provided in appendix table 5.5

The group differences in parent report of HRQL for the different lipoma groups were statistically significant and are provided in Table 5.4.

Table 5.4 PedsQL parent scores by 3 LSL groups.

The Kruskal Wallis Test demonstrates statistically significant differences in parent ratings of HRQL across LSL types, in the psychosocial scales and the Total, but not the physical scale; consequently, Mann- Whitney analysis was used which showed the caudal and dorsal groups did not differ from each other, but differences did approach significance for the School domain. The transitional group scored lower in the majority of domains than either the dorsal or the caudal group.

Statistical analysis of PedsQL Parent scores by 3 LSL groups								
	Kruskal-Wallis		Mann-Whitney		Mann-Whitney		Mann-Whitney	
	df=2, n=54		Caudal-Dorsal		Caudal-Transitional		Dorsal-Transitional	
	χ^2	*p*	U	*p*	U	*p*	U	*p*
Total	6.45	0.04	128.0	0.61	108.5	0.02	91.5	0.05
Physical	4.53	0.10	-	-	-	-	-	-
Psychosocial	7.17	0.03	123.5	0.51	99.0	0.01	96.0	0.07
Emotional	6.09	0.05	137.0	0.86	109.5	0.02	94.0	0.06
Social	6.14	0.05	135.0	0.79	124.0	0.06	80.5	0.02
School	6.43	0.05	92.0	0.07	104.5	0.02	134.5	0.60

p ≤0.01 p≤0.05

The scatterplot (figure 5.6) depicts the PedsQL child and parent Total scores for the LSL group and normative values (Upton et al., 2005).

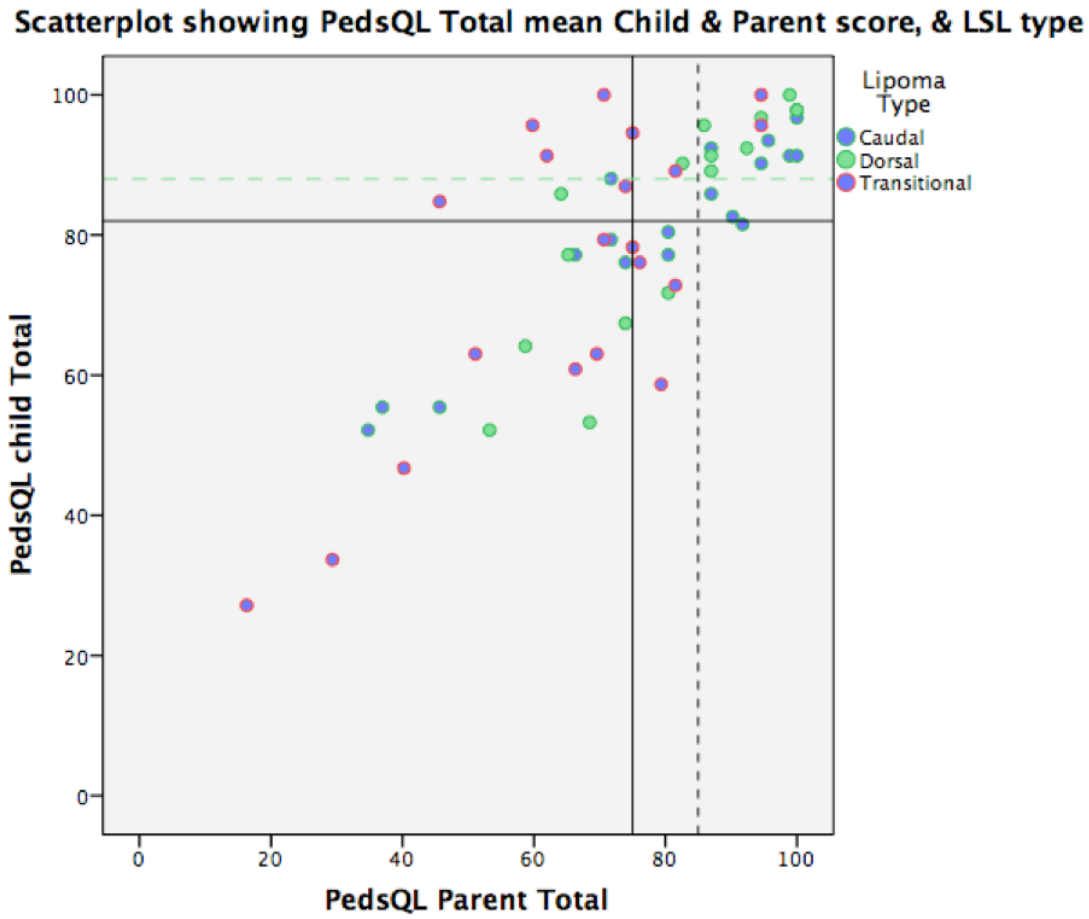


Figure 5.6 Scatterplot depicting PedsQL Total self and parent ratings for LSL and normative values.

The x axis shows PedsQL Total mean parent score: the continuous line depicts the Total mean parent score for LSL, the dotted line depicts the Total mean parent score for normative values.

The x axis shows the PedsQL Total mean child score: the continuous line depicts the Total mean child score for LSL, the dotted line shows the Total mean child score for normative values. The chart shows that many of LSL ratings are similar to those rated by the healthy population and although there is a variable assessment for all three lipoma types, there is a trend for the dorsal group to be closer to the normative values than the transitional and caudal groups. Some of the very low ratings depict scores of parents and children in the transitional group.

5.5.1.5. PedsQL and NEM ratings

The relationships between PedsQL scores and NEM ratings were evaluated using Spearman's Rank Order correlations and the results are displayed in Table 5.5. The results are provided in the table legend.

Table 5.5 PedsQL and NEM ratings.

There were statistically significant correlations between NEM Motor function and all parent PedsQL scores, and all of the child reported scores with the exception of Emotional functioning.

There were also significant correlations between NEM Sensory function and PedsQL ratings. These included all except the Emotional scale on parent report and the Total, Physical and School scales for child-report.

In contrast, for NEM Urology, the only statistically significant correlation was the parent report of social functioning. None of the correlations with NEM Bowels were significant, although the association between NEM Bowels and child reported social functioning approached significance.

PedsQL and NEM ratings (n=54)										
	NEM ratings									
	Motor		Sensory		Urology		Bowels		NEM Total	
	r_s	p	r_s	p	r_s	p	r_s	p	r_s	p
Child										
Total	0.45	0.001	0.34	0.01	0.04	0.80	-0.20	0.16	0.22	0.12
Physical	0.51	<0.001	0.43	0.001	0.09	0.53	-0.09	0.51	0.32	0.02
Psychosocial	0.37	0.005	0.27	0.05	0.07	0.64	-0.21	0.14	0.19	0.17
Emotional	0.21	0.136	0.15	0.27	0.05	0.73	-0.21	0.13	0.10	0.49
Social	0.42	0.002	0.20	0.16	0.17	0.23	-0.24	0.08	0.17	0.21
School	0.29	0.032	0.27	0.05	0.05	0.73	-0.03	0.83	0.19	0.17
Parent										
Total	0.53	<0.00	0.42	0.002	0.17	0.21	-0.04	0.78	0.36	0.008
Physical	0.52	<0.00	0.46	0.001	0.22	0.13	0.05	0.74	0.44	0.00
Psychosocial	0.46	<0.00	0.36	0.007	0.16	0.26	-0.09	0.53	0.28	0.04
Emotional	0.42	0.002	0.20	0.15	0.13	0.37	-0.11	0.42	0.26	0.06
Social	0.42	0.002	0.40	0.003	0.27	0.05	-0.04	0.76	0.33	0.02
School	0.32	0.020	0.29	0.03	0.00	0.98	-0.13	0.37	0.07	0.64

$p \leq 0.01$ $p \leq 0.05$

5.5.1.6. PedsQL and Physical activity (PAQ)

As described in the methodology chapter (chapter 3), the PAQ is a self-administered scale which measures participation in sporting activities over the previous 7 days and categorises children into groups of those who are "sufficiently active" and "not at risk" (from ill health derived from inactivity), and those who are "low active" and "at risk. The results are shown in Table 5.6.

Table 5.6 PedsQL and PAQ score

There were no significant correlations between the PedsQL, child or parent-report and the child form of the PAQ. The only significant correlation between PedsQL child report and the adolescent PAQ-A was on the Physical scale as expected. There were several significant correlations between PedsQL parent report and the PAQ-A, including both the Physical and Emotional scales and the Total score.

	PAQ-C (n=11)		PAQ-A (n=23)		PAQ-C/A (n=34)	
	rs	p	rs	p	rs	p
Child						
Total	0.15	0.67	0.41	0.05	0.35	0.04
Physical	0.05	0.89	0.42	0.05	0.33	0.05
Psychosocial	0.16	0.64	0.36	0.09	0.28	0.11
Emotional	-0.06	0.87	0.27	0.21	0.17	0.33
Social	-0.02	0.95	0.35	0.10	0.25	0.16
School	0.20	0.56	0.22	0.31	0.22	0.21
Parent						
Total	0.21	0.54	0.49	0.02	0.34	0.05
Physical	0.05	0.88	0.55	0.01	0.40	0.02
Psychosocial	0.10	0.77	0.36	0.09	0.27	0.13
Emotional	0.08	0.81	0.40	0.05	0.30	0.09
Social	0.12	0.72	0.32	0.14	0.24	0.17
School	0.13	0.70	0.25	0.25	0.19	0.29

p ≤0.01 p≤0.05

5.5.1.6.1. PedsQL and pain

5.5.1.6.1.1. PedsQL and NEM sensory, and PedsQL and PedsQL PPQ pain scale (as described in chapter 3, the PedsQL PPQ scale displays a 100mm horizontal line ranging from scores of 0 (no pain) to 10 (worst pain)).

Results from the analysis between PedsQL and NEM sensory, and PedsQL and PedsQL PPQ pain scale are shown in Table 5.7.

Table 5.7 PedsQL and NEM sensory and PedsQL PPQ

There were significant correlations between PedsQL scores and NEM sensory ratings. These included all except the Emotional scale on parent report, and the Total, Physical and School scales on child report.

Correlation between the PedsQL and PPQ showed uniformly negative correlations consistent with decreasing HRQL with increasing pain, and these relationships were statistically significant for both child report and for parent report for Total and Physical HRQL. Relationships between pain and School domain of the PedsQL and between pain and overall Psychosocial function were either at, or close to significance for both children and parents.

	PedsQL & NEM Sensory	PedsQL & NEM Sensory	PedsQL PPQ	PedsQL PPQ
	r_s	p	rs	p
Child				
Total	0.34	0.01	0.38	0.004
Physical	0.43	0.001	0.44	0.001
Psychosocial	0.27	0.05	-0.29	0.04
Emotional	0.15	0.27	-0.21	0.13
Social	0.20	0.16	-0.18	0.18
School	0.27	0.05	-0.26	0.06
Parent				
Total	0.42	0.02	0.37	0.006
Physical	0.46	0.01	-.51	<0.001
Psychosocial	0.36	0.007	-0.265	0.06
Emotional	0.20	0.15	-0.31	0.02
Social	0.40	0.003	-0.23	0.09
School	0.29	0.03	-0.23	0.09

p ≤0.01 p≤0.05

5.5.1.6.1.2. PedsQL and binary pain heading

The relationship between PedsQL scores and binary pain status (whether the child did, or did not, report pain) is shown in Figure 5.7 and table 5.8.

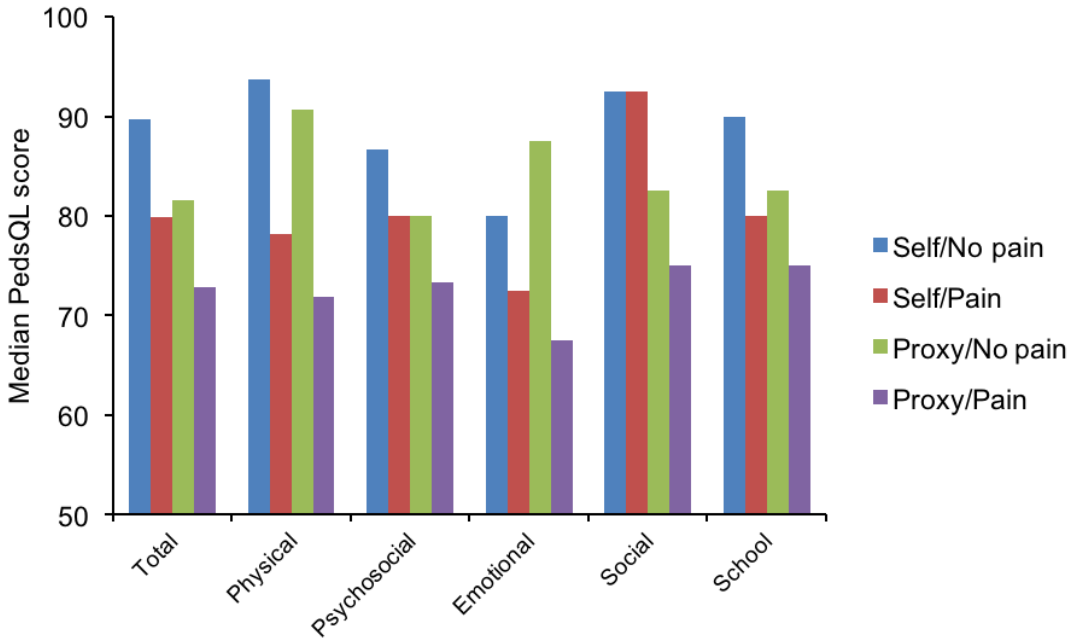


Figure 5.7 PedsQL scores by binary pain status

The x axis shows the PedsQL domains and the Total score, the y axis the median score (maximum score = 100). The results show that the presence of pain tended to be associated with lower scores in almost all PedsQL domains on both child (self)-and parent (proxy) reports.

Table 5.8 Comparison of PedsQL scores for presence / absence of pain

There was a significant association between the child and parent PedsQL ratings on the physical subscale and the presence of pain. The Total score was significant for child -report and approached significance for parent report. There was also a significant association between Emotional HRQL on parent report.

Comparison of PedsQL scores for absence or presence of pain								
	No pain (n=26)			Pain (n=28)			Mann-Whitney	
Child	n	Median	IQR	n	Median	IQR	U	*p*
Total	26	89.67	19.84	28	79.89	33.97	248.0	0.04
Physical	26	93.75	14.06	28	78.13	35.94	189.5	0.002
Psychosocial	26	86.67	22.92	28	80.00	32.50	284.0	0.17
Emotional	26	80.00	40.00	28	72.50	42.50	308.5	0.33
Social	26	92.50	22.50	28	92.50	40.00	321.5	0.44
School	26	90.00	30.00	28	80.00	43.75	307.5	0.32
Parent							U	*p*
Total	26	81.52	28.53	28	72.82	36.41	257.5	0.07
Physical	26	90.63	32.03	28	71.88	46.88	225.5	0.02
Psychosocial	26	80.00	27.08	28	73.33	28.75	287.0	0.18
Emotional	26	87.50	40.00	28	67.50	37.50	250.0	0.05
Social	26	82.50	36.25	28	75.00	42.50	333.5	0.59
School	26	82.50	30.00	28	75.00	37.50	332.5	0.58

p ≤0.01 p≤0.05

Table 5.9 and Table 5.10 display the results from all PedsQL analysis.

Table 5.9 PedsQL child data analysis

The physical domain of the PedsQL showed more statistically significant correlations with other variables, than other any of the other PedsQL domains.

PedsQL child						
	Physical	Psychosocial	Emotional	Social	School	Total
NEM urology						
NEM bowels						
NEM sensory						
NEM motor						
NEM total						
Pain binary						
Pain PPQ						
PAQ-C						
PAQ-A						
PAQ-C/A						

p ≤0.05 p≤0.1 ; No association

Table 5.10 PedsQL parent and data analysis

The PedsQL parent ratings are displayed on the horizontal axis and the NEM variables, pain and PAQ activity levels along the y axis. The physical domain of the PedsQL showed more statistically significant correlations with other variables, than other any of the other PedsQL domains.

PedsQL parent						
	Physical	Psychosocial	Emotional	Social	School	Total
NEM urology						
NEM bowels						
NEM sensory						
NEM motor						
NEM total						
Pain binary						
Pain PPQ						
PAQ-C						
PAQ-A						
PAQ-C/A						

p ≤0.05 p≤0.1 ; No association

5.5.1.7. Summary of PedsQL

- Children rated their HRQL as similar to the healthy population with the exception of the Physical domain; parents rated their child's HRQL as lower than the healthy population in all domains, although the ratings of Emotional and School domains were not of statistical significance.

- Females self-reported higher HRQL than males, parent ratings tended to be lower than self-report for females on all scales. The same trend was also apparent for males on most scales.
- The lowest scores of HRQL were in children in the transitional group. Child ratings showed the dorsal group to have the highest HRQL whereas parent ratings showed the caudal group to have the highest HRQL.
- PedsQL parent and child ratings showed significant correlation with the Motor and Sensory domains of the NEM and the Total score; there were no correlations with Bowel function. Urology parent ratings correlated with the Social domain.
- Physical activity as measured on the PAQ-A, showed correlations with the child rating score for the Physical domain only, Parent PedsQL ratings correlated with the Physical and Emotional domains.
- Increased child and parent pain ratings correlated with decreased Physical and Total scores of the PedsQL. Pain also correlated with Psychosocial and School domains for child and parent ratings.
- PedsQL child and parent ratings indicated that physical HRQL was most impaired, with this being particularly evident in the presence of pain.

5.5.2. The Child Health Questionnaires (CHQ)

Heath-related quality of life was also measured using two versions of the Child Health Questionnaire: a 50-item parent report form (CHQ-PF50) and an 87-item child self-report version (CHQ-CF87). The publishers provided normative data for the CHQ-PF50 and normative data for the CHQ-CF87 was taken from Raat et al (Raat et al., 2002).

5.5.2.1. CHQ-PF50

The CHQ-PF50 comprises 10 domain-specific scales (as described in the Methodology chapter). According to standard procedures for the CHQ-CF50, z-scores were calculated for each scale using the normative data, to analyse if a particular score was equal to the mean score. Using standard algorithms, two

composite scores, the Physical Summary Score (PhS) and the Psychological Summary score (PsS), were calculated.

Tests of normality indicated that distributions of all but one of the scales violated assumptions of normality, generally indicating negative skewness (appendix 5.6). Composite scores approximated sufficiently to a normal distribution to allow parametric analyses.

As for previous measures of child psychosocial functioning (PedsQL), supplementary parametric analyses of the domain scores are reported in appendix table 5.7.

5.5.2.1.1. Comparison of CHQ-PF50 data to normative data

The obtained CHQ-PF50 scale scores were compared to the normative median value of z (by definition z = 0). Test of the statistical significance of the differences is shown in appendix table 5.8. Figure 5.8 displays the median CHQ-PF50 z scores.

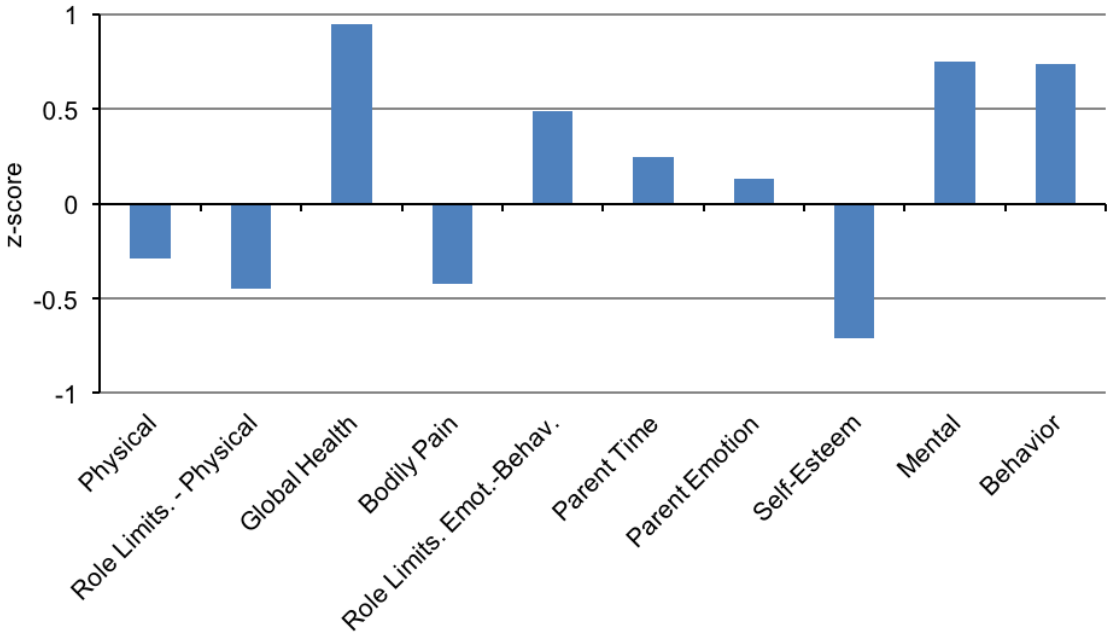


Figure 5.8 Median CHQ-PF50 z-scores

The x axis shows the CHQ-PF50 domains; the y axis shows the z scores (the normative median value = 0). The graphs show higher than average ratings in the area of Global Health, Mental Health and Behaviour and lower than average ratings in the area of Physical, Bodily Pain and Self Esteem.

5.5.2.1.2. CHQ-PF50 and Gender

There was no effect of gender of CHQ-PF50 domains (Mann-Whitney U Test). The statistical analysis is provided in appendix table 5.9.

5.5.2.1.3. CHQ-PF50 and LSL type

Figure 5.9 shows the median CHQ-PF50 scale scores for each LSL type.

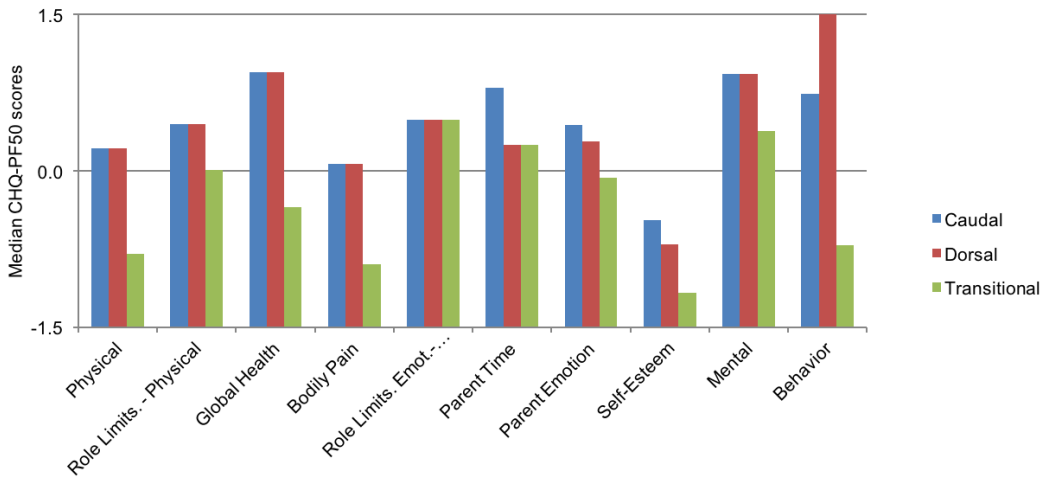


Figure 5.9 Median CHQ-PF50 scores by LSL type

The x axis displays the domains of the CHQ-PF50 and the y axis displays the Median CHQ-PF50 scores indicates a trend on most scales towards lower scores associated with transitional LSLs. The exceptions were very similar ratings of Emotional/Behavioural role limitations for all 3 LSL types, and a similarity between Dorsal and Transitional LSL for Parent Time.

Appropriate non-parametric or parametric tests were used to analyse the effect of LSL type on CHQ-PF50 scores.

There were statistically significant differences in Bodily Pain ($x^2=11.72$, $p=0.003$) Parent Time ($x^2=11.72$, $p=0.003$) and Behaviour ($x^2=8.8070$, $p=0.018$) using the Kruskal-Wallis Test. Therefore, these 4 variables were subjected to post-hoc Mann-Whitney U-tests which indicated that there were no significant differences between caudal and dorsal cases, but both groups were significantly different from transitional cases in terms of Global Health and Bodily Pain.

For Parent Time, only the difference between caudal and transitional cases was statistically significant ($u=96, p=0.005$). In contrast, for Behaviour, the dorsal-transitional comparison was significant ($u=72$, $p=0.007$), although the caudal-transitional comparison was close to significance ($u=125$, $p=0.05$).

One-way ANOVA indicated the presence of statistically significant group differences on the Physical (F=3.38, p=0.04) but not the Psychological (F=2.24, p=0.117) composite. Regarding the former, post-hoc testing suggested that scores for caudal and dorsal cases were higher than those for transitional cases, but both comparisons failed to reach significance. There was a trend for the transitional group to require more Parent Time, have increased Bodily Pain and reduced Global Health. The statistical analysis is provided in appendix table 5.10.

5.5.2.1.4. CHQ-PF50 and NEM ratings

The relationship between CHQ-PF50 and NEM variables was evaluated using Spearman's Rank Order Correlations (Table 5.11)

Table 5.11 CHQ-PF50 and NEM rating

The x axis displays the NEM ratings, the y axis displays the CHQ-PF50 domains. NEM motor and sensory ratings correlated significantly with the majority of CHQ-PF50 domains, with a significant correlation between Parent Time and NEM Motor, but not NEM Sensory. NEM urology correlated significantly with Bodily Pain Self-Esteem and Mental Health. NEM Bowel only correlated significantly with Self-Esteem. PhS* Physical summary Score; PsS**Psychological summary Score.

CHQ-PF50 and NEM ratings										
	NEM ratings									
	Motor		Sensory		Urology		Bowels		NEM Total	
	r_s	p	r_s	p	r_s	p	r_s	p	r_s	p
Physical Functioning	0.52	<0.01	0.48	<0.001	0.22	.12	-0.13	0.35	0.36	0.007
Role Limitations - Physical	0.32	0.017	0.24	.084	0.16	.25	0.07	0.63	0.32	0.02
Global Health	0.17	0.22	0.09	.543	0.26	.06	0.01	0.93	0.24	0.08
Bodily Pain	0.31	0.02	0.37	.007	0.49	<0.001	0.26	0.06	0.61	<0.001
Role Limits. - Emot./Behav.	0.30	0.03	0.30	.028	0.09	0.54	-0.24	0.08	0.17	0.21
Parent Time	0.33	0.02	0.22	.116	0.15	0.28	-0.20	0.15	0.21	0.13
Parent Emotion	0.05	0.70	0.04	.776	0.14	0.32	0.002	0.99	0.12	0.39
Self-Esteem	0.06	0.66	0.08	.592	0.28	0.04	0.29	0.03	0.28	0.04
Mental	0.28	0.04	0.34	.011	0.07	0.63	0.12	0.41	0.39	0.01
Behaviour	0.10	0.50	-0.11	.427	0.35	0.01	0.18	0.20	0.33	0.02
PhS*	0.52	<0.001	0.43	.001	0.27	0.05	-0.06	0.65	0.43	0.001
PsS**	0.20	0.16	0.05	.706	0.23	0.09	0.11	0.41	0.32	0.02

p ≤0.01 p≤0.05

5.5.2.1.5. CHQ-PF50 and Physical Activity score (PAQ)

Correlations between PAQ-C ratings and CHQ-PF50 scales were uniformly low and none approached statistical significance (Spearman's Rank Order correlation). PAQ-A ratings were significantly correlated with the CHQ-PF50 Physical Functioning only (r=0.053, p=0.01), while the association with Self-Esteem marginally fell short of this (r=0.35, p=0.08). The results are provided in appendix table 5.11.

5.5.2.1.6. CHQ-PF50 and pain

5.5.2.1.6.1. CHQ-PF50 and NEM sensory and CHQ-PF50 and PPQ

The relationship between CHQ -PF50 scores and NEM sensory ratings and the PPQ scores were evaluated as shown in Table 5.12

Table 5.12 CHQ PF 50 and NEM sensory and PedsQL PPQ

There were several significant positive correlations between CHQ-PF50 scores and NEM sensory ratings, and between parent PedsQL PPQ pain ratings and NEM scores. PhS* Physical summary Score; PsS**Psychological summary Score

	CHQ-PF50 & NEM Sensory	CHQ-PF50 & NEM Sensory	PedsQL PPQ	PedsQL PPQ
	rs	p	rs	p
Physical Functioning	0.48	<0.001	-0.45	0.001
Role Limitations - Physical	0.24	0.84	-0.26	0.06
Global Health	0.09	.543	-0.15	0.27
Bodily Pain	0.37	0.007	-0.63	<0.001
Role Limits. - Emot./Behav.	0.30	.028	-0.39	0.004
Parent Time	0.22	.116	-.34	0.01
Parent Emotion	0.05	.776	-.29	0.03
Self-Esteem	0.08	.592	-0.09	0.53
Mental	0.34	0.011	-0.31	0.02
Behaviour	-0.11	.427	-0.10	0.49
PhS*	0.43	.001	-0.51	<0.001
PsS**	0.43	.706	-0.23	0.09

p ≤0.01 p≤0.05

5.5.2.1.6.2. CHQ-PF50 and binary measure of pain

As expected, children classified as experiencing pain were rated by parents as having higher levels of pain on CHQ-PF50 Bodily Pain scale than those who were classified as not experiencing pain. Binary rating of pain also differentiated between cases in terms of Parent Emotion and Mental Health. The results are provided in appendix table 5.12.

5.5.2.1.6.3. CHQ-PF50 Bodily pain scale and LSL type

There was a highly significant difference in bodily pain scores between the three groups of lipoma with children in the transitional group reporting more pain and impact of pain than the caudal group and more than the dorsal group. The results are provided in appendix table 5.13.

5.5.2.1.6.4. CHQ-PF50 Bodily pain scale and PAQ activity scale

There were no statistically significant associations between the CHQ-PF50 Bodily pain scales and activity as measured on the PAQ. The results are provided in appendix table 5.14.

Table 5.13 provides a summary of CHQ-PF50 analysis.

Table 5.13 Summary of CHQ-PF50

The CHQ-PF50 ratings are displayed on the horizontal axis and the NEM variables, pain and PAQ activity levels along the y axis. Physical Functioning and Bodily Pain correlated significantly with the NEM variables, pain and activity scores, more than any of the other variables.

PF Physical Functioning; RP Role limitations-physical; GH Global Health; BP Bodily Pain; Re Role limitations- emotional; PT Parent Time; PE Parent emotion; SE Self Esteem; MH Mental Health; BE Behaviour; PhS Physical summary Score; PsS Psychological summary Score PsS

	PF	RP	GH	BP	Re	PT	PE	SE	MH	BE	PhS	PsS
NEM urology												
NEM bowels												
NEM sensory												
NEM motor												
NEM total												
Pain binary												
Pain PPQ												
PAQ-C												
PAQ-A												
PAQ-C/A												

p ≤0.05 p≤0.1 ; No association

5.5.2.1.7. Summary of CHQ-PF50

- CHQ-PF50 scores indicated higher than average ratings compared to the normative population in the area of Global Health, Mental Health and General Behaviour; in contrast, Physical Functioning and Self-Esteem were below the median for healthy normative values.
- There were no significant effects of gender on the CHQ-PF50.
- The transitional group was identified as having the lowest ratings in the majority of domains; ratings were similar between the caudal and dorsal groups in the majority of domains.
- NEM motor and sensory ratings correlated significantly with the majority of CHQ-PF50 domains, urology with four domains and bowels with none.
- Physical activity as measure by the PAQ indicated a significant correlation with the CHQ-PF50 Physical Functioning domain only. There were no statistically significant findings between the CHQ-PF50 Bodily pain scales and activity as measured on the PAQ.
- Pain correlated with many of the CHQ-PF50 domains and occurred more commonly in the transitional group; the caudal and dorsal group had similar ratings in the majority of domains.

5.5.2.2. CHQ-CF87

The CHQ-CF87 comprises 14 domain-specific variables (as described in the methodology chapter).

Tests of normality indicated that distributions of all but one of these variables significantly violated assumptions of normality, and all showed negative skewness (appendix table 5.15). Consequently, non-parametric methods were used to analyse the data.

5.5.2.2.1. Comparison of CHQ-CF87 data to normative data

The patient data were compared to the normative data reported by Raat et al (Raat et al., 2002). It is noted that Raat et al only reported selected scales. As in

previous analyses, their means were assumed to be an approximation of the normative median for the purposes of non-parametric analyses.

However, for several scales, an alternative estimate of the median was afforded by Raat et al reporting the percentage of respondents reporting the best possible score. It follows that, where this percentage exceeds 50%, the median must be the maximum. From this perspective it can be seen that there are pronounced negative skews on at least several of the scales, and that the means underestimated medians. Where this was evident, LSL medians were compared with the maximum value of the scale. The results are provided in appendix table 5.16.

The results of One-Sample Wilcoxon Signed Rank tests are presented in figure 5.10 and table 5.14. Z scores were not used, as it was more appropriate to compare the data with the scores reported by Raat et al (Raat et al., 2002).

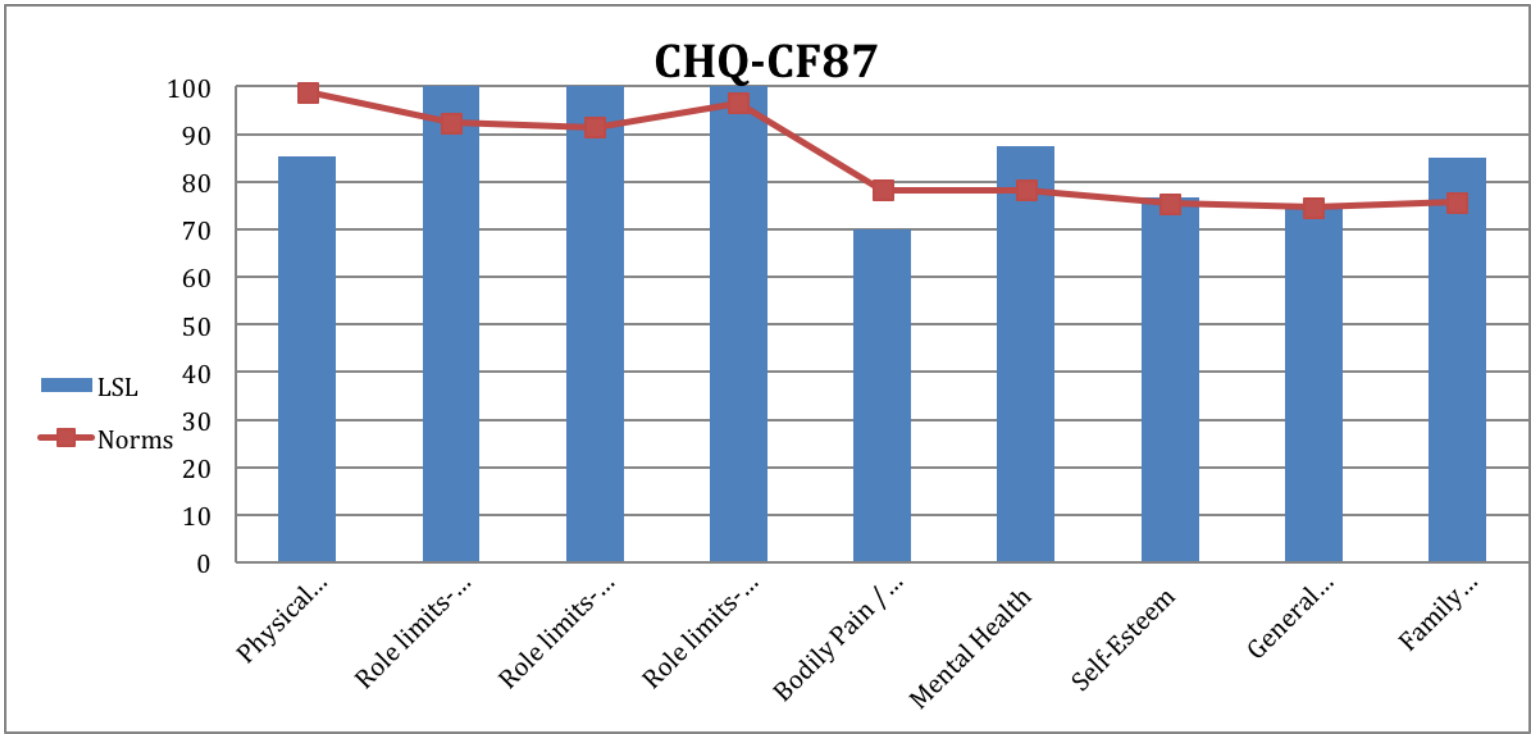


Figure 5.10 CHQ-CF87 LSL and normative values.

The x axis displays the CHQ-CF87 domains; the y axis displays the score (maximum score = 100) achieved for each domain. In comparison to the general population, children with LSL scored significantly lower in Physical Functioning when compared to normative values, but higher in many of the other scales.

Table 5.14 Comparison of LSL and healthy normative CHQ-CF87 data (Raat et al, 2002)

The x axis displays the LSL and normative values, and the y axis the CHQ-CF87 domains. The results identify that LSL children reported significantly lower ratings of Physical Functioning, Role Limits- Behavioural, and Bodily Pain /Discomfort than normative values.

	LSL		Normative		1-sample Wilcoxon
CHQ-CF87	Median	IQR	Est. median	IQR	*p*
Physical Functioning	85.19	37.96	96.8	4	0.001
Role Limits. - Emotional	100.00	13.89	92.3	11	0.61
Role Limits. - Behavioural	100.00	0.00	91.4	0	<0.001
Role Limits. - Physical	100.00	33.33	96.5	0	0.91
Bodily Pain/Discomfort	70.00	30.00	78.2	30	0.004
Mental Health	87.50	23.28	78.2	15	0.26
Self-Esteem	76.79	35.71	75.4	16	0.40
General Health Perceptions	74.79	29.17	74.6	21	0.42
Family Cohesion	85.00	40.00	75.7	40	0.07

p ≤0.01 p≤0.05

A discussion of statistical reasons for using means / medians in analysing the CHQ-CF87 is provided in Appendix 5.17.

5.5.2.2.2. CHQ-CF87 and Gender

The effect of gender on CHQ-CF87 scores indicated that females reported significantly more Bodily Pain/Discomfort (i.e. a lower score) than males (u=37,

p=0.045, Mann Whitney U Test). No other comparisons were statistically significant (appendix table 5.18).

5.5.2.2.3. CHQ-CF87 and LSL type

Figure 5.11 shows the median CHQ-CF87 scale scores for each LSL type. Note that the Change in Health ratings, where the maximum score was 5, were multiplied by 20 to fit to a similar scale as the standardised scores.

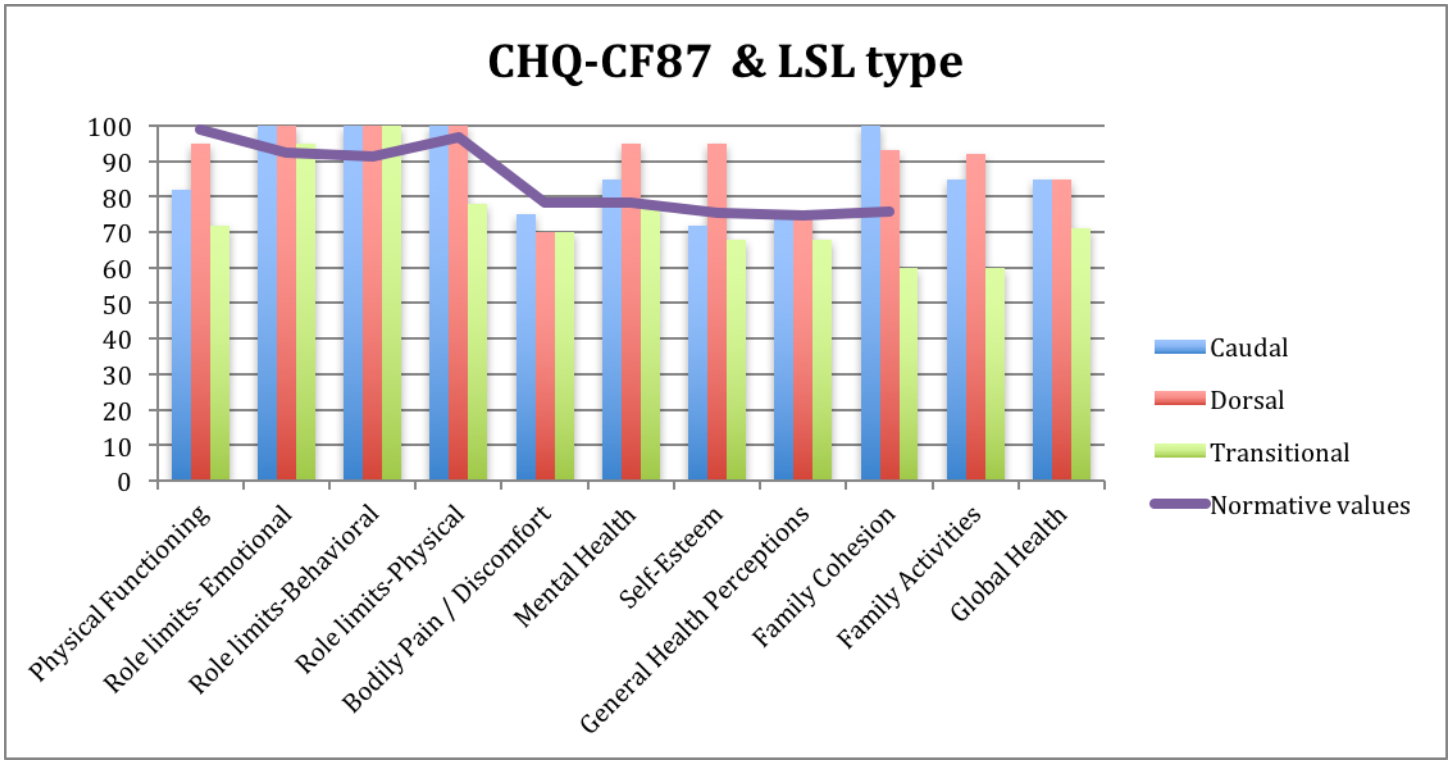


Figure 5.11 CHQ-CF87 & LSL type

The x axis shows the CHQ-CF87 domains, the y axis the total score obtained for each domain (maximum score = 100). The results show median scores at the ceiling for caudal and dorsal types on the three Role Limitations scales; report of dorsal cases tended to have the highest scores and transitional reports the lowest on most measure. There are no measure provided by Raat el al for Family Activities and Global Health (Raat et al., 2002).

There was no statistical association between LSL type and any of the CHQ-CF87 domains and the statistical analysis is provided in appendix 5.19.

5.5.2.2.4. CHQ-CF87 and NEM ratings

The relationship between CHQ-CF87 and NEM variables was evaluated using Spearman's Rank Order Correlations and displayed in table 5.15.

Table 5.15 CHQ-CF87 and NEM ratings.

There were several correlations between the Sensory, Motor and Total scores and the CHQ-CF87 scores. NEM Bowels did not correlate significantly with any CHQ-CF87 scores and NEM Urology only correlated significantly with CHQ-CF87 Mental Health. Rle;Role Limits. – Emotional; RLe Role limits behavioural; Rlp Role Limits. – Physical; Bpd-Bodily Pain/ Discomfort; General Health Percept-Ghp

CHQ-CF87 and NEM rating										
	NEM ratings									
	Motor		Sensory		Urology		Bowels		NEM Total	
	rs	p	rs	p	rs	p	rs	p	rs	p
Global Health	0.36	0.07	0.31	0.14	0.15	0.49	-0.07	0.74	0.28	0.17
Physical Functioning	0.60	0.001	0.61	0.000	0.10	0.64	0.15	0.45	0.47	0.016
Rle	0.04	0.83	0.41	0.04	0.05	0.79	-0.11	0.58	0.15	0.47
Rlb. -	-0.02	0.93	0.06	0.76	0.08	0.70	-0.20	0.36	-0.09	0.67
Rlp	0.50	0.01	0.45	0.02	0.23	0.26	0.01	0.97	0.44	0.03
Bpd	0.26	0.21	0.28	0.17	-0.07	0.73	0.06	0.76	0.09	0.67
Behaviour	0.35	0.08	0.47	0.01	0.37	0.06	0.08	0.70	0.40	0.04
Global Behaviour Item	0.24	0.24	0.14	0.51	-0.11	0.59	-0.26	0.19	0.01	0.97
Mental Health	0.28	0.16	0.32	0.11	0.41	0.04	-0.01	0.98	0.31	0.13
Self-Esteem	0.48	0.01	0.47	0.01	0.48	0.01	-0.20	0.31	0.43	0.03
GHp	0.52	0.007	0.23	0.25	0.16	0.44	-0.13	0.52	0.28	0.17

CHQ-CF87 and NEM rating										
	NEM ratings									
	Motor		Sensory		Urology		Bowels		NEM Total	
Change in Health	0.29	0.15	0.32	0.11	0.16	0.44	-0.31	0.12	0.20	0.33
Family Activities	0.66	0.0003	0.44	0.02	0.37	0.06	-0.03	0.90	0.52	0.007
Family Cohesion	0.520	0.008	0.32	0.12	0.11	0.59	-0.10	0.63	0.24	0.25

p ≤0.01 p≤0.05

5.5.2.2.5. CHQ-CF87 and Physical Activity

Correlations between ratings on the CHQ-PF87 and child and adolescent forms of the PAQ (PAQ-C and PAQ-A respectively) were undertaken using Spearman's Rank Order.

It was noted that, given the limited overlap in age ranges of the CHQ-CF87 and PAQ-C, only 3 participants had completed both. In this context, there was a perfect negative correlation between the two measures. However, given the small sample size, the statistical significance of this finding could not be determined. No other correlations were significant. The results are displayed in table 5.16.

Table 5.16 CHQ-CH87 and PAQ

PAQ-A ratings were significantly correlated with the CHQ-CF87 Physical Functioning and Role Limitations – Physical scales. In addition, there were significant associations with the majority of other CHQ-CF87 scales and the relationship with the Global Behaviour Item and Change in Health approached significance. The pattern of significant correlations between CHQ-CF87 and combined PAQ-C/A ratings was similar in most respects. However, differences on Global Health and Mental Health did not reach significance.

CHQ- **and PAQ**	PAQ-C			PAQ-A			PAQ-C/A		
	n	rs	*p*	*n*	rs	*p*	*n*	rs	*p*
Global Health	3	-1.0	n/a	20	0.47	0.04	23	0.33	0.12
Physical Functioning	3	0.87	0.33	21	0.59	0.005	24	0.63	0.001
Role Limits. – Emotional	3	0.50	0.67	21	0.33	0.15	24	0.34	0.11
Role Limits. – Behavioural	3	n/a	n/a	21	0.13	0.58	24	0.11	0.62
Role Limits. – Physical	3	0.50	0.67	21	0.59	0.01	24	0.60	0.002
Bodily Pain/Discomfort	3	0.87	0.33	21	-0.08	0.74	24	0.07	0.74
Behaviour	3	0.00	1.00	21	0.45	0.04	24	0.44	0.03
Global Behaviour Item	3	0.50	0.67	21	0.42	0.06	24	0.36	0.09
Mental Health	3	-0.87	0.33	21	0.50	0.02	24	0.38	0.07
Self-Esteem	3	-0.50	0.67	21	0.66	0.001	24	0.51	0.01
General Health Perceptions	3	-0.87	0.33	21	0.77	<0.001	24	0.62	0.001

CHQ- **and PAQ**	PAQ-C			PAQ-A			PAQ-C/A		
Change in Health	3	-0.87	0.33	21	0.39	0.08	24	0.27	0.20
Family Activities	3	-0.87	0.33	21	0.70	<0.001	24	0.59	0.002
Family Cohesion	3	0.50	0.67	20	0.45	0.04	23	0.43	0.04

p ≤0.01 p≤0.05

5.5.2.2.6. CHQ-CF87 and Pain

The relationship between CHQ-CF 87 scores and NEM sensory ratings and the PedsQL PPQ scores are shown in table 5.17.

Table 5.17 CHQ-CF 87 and pain ratings

NEM Sensory scores correlated with six CHQ-CF87 variables and four PedsQL PPQ ratings; both the NEM sensory and the PedsQL PPQ correlated with Physical Functioning, Self Esteem and Family Activities domains of the CHQ-CF87.

	CHQ-CF87 & NEM Sensory	CHQ-CF87 & NEM Sensory	PedsQL PPQ	**PedsQLPPQ**
	r_s	p	rs	p
Global Health	0.31	0.14	-0.12	0.55
Physical Functioning	0.61	0.00	-0.62	0.001
Role Limits. - Emotional	0.41	0.04	-0.35	0.08
Role Limits. - Behavioural	0.06	0.76	-0.33	0.10
Role Limits. - Physical	0.45	0.02	-0.31	0.13
Bodily Pain/ Discomfort	0.28	0.17	-0.49	0.01
Behaviour	0.47	0.01	-0.27	0.19
Global Behaviour Item	0.14	0.51	-0.12	0.55
Mental Health	0.32	0.11	-0.24	0.24
Self-Esteem	0.47	0.01	-0.41	0.04
General Health Percept	0.23	0.25	-0.14	0.49
Change in Health	0.32	0.11	-0.01	0.96
Family Activities	0.44	0.02	-0.40	0.04
Family Cohesion	0.32	0.12	-0.25	0.22

p ≤0.01 p≤0.05

5.5.2.2.6.1. CHQ-CF 87 and binary pain scores

Children classified as experiencing pain did not reported statistically significant more Bodily Pain/Discomfort on the CHQ-CF 87. However, there were statistically negative correlations between pain and Physical Functioning (U=39; p=0.02) and Role Limits- Physical (U=48; p=0.02).

5.5.2.2.6.2. CHQ-CF87 Bodily pain scales and PAQ scores

There were no statistically significant findings between the CHQ-CF87 Bodily pain scales and activity as measured on the PAQ (table 5.16).

5.5.2.2.6.3. CHQ-CF87 Bodily pain scale and LSL type

There were no statistically significant findings between the CHQ-CF87 Bodily pain scales and LSL type (appendix table 5.19).

The results from all CHQ-CG87 data are displayed in Table 5.18.

Table 5.18 The results from all CHQ-CF87 correlations

The x axis displays the CHQ-CH87 domains, the y axis the NEM, pain and activity ratings. Physical Functioning and self-esteem correlated significantly with the NEM variables, pain and activity scores, more than any of the other variables. GH General Health; Re Role limitations- emotional; PF Physical Functioning; Rb Role limitations- behavioural; Rp Role limitations- physical; BP Bodily Pain; Be Behaviour; GB Global Behaviour item; MH Mental Health; SE Self Esteem; GHP General Health Perception ; CiH Changes in Health; FA Family Activity; FC Family Cohesion.

	GH	Re	PF	Rb	Rp	Bp	Be	GB	MH	SE	GHP	CiH	FA	FC
NEM urology														
NEM bowels														
NEM sensory														
NEM motor														
NEM total														
Pain binary														
Pain PPQ														
PAQ-C														
PAQ-A														
PAQ-C/A														

p ≤0.05 p≤0.1 ; No association

5.5.2.2.7. Summary of CHQ-CF87.

- In comparison to the general population, children with LSL scored significantly lower in the Physical Functioning domain when compared to normative values, but higher on many of the other scales.
- The effect of gender on CHQ-CF87 scores indicated that females reported significantly more Bodily Pain/Discomfort than males.
- The Transitional group reported the lowest scores on most measures of the CHQ-CF87 and the dorsal group the highest.
- There were several correlations between the NEM Sensory, Motor and Total scores and the CHQ-CF87 scores. NEM Bowels did not correlate significantly with any CHQ-CF87 scores and NEM Urology only correlated significantly with CHQ-CF87 Mental Health.
- PAQ-A ratings were significantly correlated with the CHQ-CF87 Physical Functioning, and Role Limitations – Physical scales. There were also significant associations with the majority of other CHQ-CF87 scales.
- NEM Sensory scores correlated with six CHQ-CF87 variables and four PedsQL PPQ ratings; both the NEM sensory and the PedsQL PPQ correlated with Physical Functioning, Self Esteem and Family Activities domains of the CHQ-CF87.
- There were statistically negative correlations between binary pain scores and Physical Functioning.
- There were no statistically significant findings between the CHQ-CF87 Bodily pain scales and activity as measured on the PAQ.
- There were no statistically significant findings between the CHQ-CF87 Bodily pain scales and LSL type.

5.5.2.2.8. Correlations between CHQ-PF50 and CHQ-CF87

The results of the degree of association between correspondingly named scales is provided in table 5.19.

Table 5.19 Correlations between CHQ-PF50 and CHQ-CF87

There is a significant correlation between parent and child ratings of Physical Functioning and between the CHQ-PF50 Physical with 8 other CHQ-CF87 scales; the CHQ-CF87 Family Activities was significantly associated with 10 of the CHQ-PF50 scales, including the two summary composites. Conversely, CHQ-CF87 Bodily Pain did not correlate significantly with any of the CHQ-PF50 variables.

: Correlations between CHQ-PF50 and CHQ-CF87. Correlations on scales of the same name are bordered

CHQ-CF87	n		CHQ-PF50 Physical	Role Limitations: Physical	Global Health	Bodily Pain	Role Limits: Emotional/ Behavioural	Time	Parent Emotion	Self-Esteem	Mental Health	Behaviour	PhS	PsS
Global Health	25	*rs*	0.51	0.32	0.34	0.13	0.29	0.40	0.15	0.12	0.16	-0.05	0.53	0.14
		p	0.01	0.12	0.09	0.55	0.16	0.05	0.48	0.58	0.43	0.82	0.01	0.50
Physical Functioning	26	*rs*	0.60	0.25	0.09	0.56	0.39	0.40	0.22	0.15	0.55	-0.20	0.56	0.17
		p	0.001	0.23	0.66	0.003	0.05	0.04	0.28	0.45	0.004	0.33	0.003	0.42
Role Limits. - Emotional	26	*rs*	0.21	0.24	0.08	0.29	0.38	0.42	0.37	0.12	0.57	-0.22	0.29	0.23
		p	0.31	0.23	0.69	0.16	0.05	0.03	0.06	0.57	0.003	0.29	0.15	0.25
Role Limits. - Behavioural	26	*rs*	0.05	0.15	0.05	0.18	0.20	-0.08	0.07	0.15	0.23	-0.05	0.08	0.11
		p	0.81	0.47	0.80	0.39	0.33	0.72	0.73	0.48	0.26	0.81	0.70	0.59
Role Limits. - Physical	26	*rs*	0.48	0.20	-0.17	0.51	0.34	0.31	0.18	0.28	0.50	0.11	0.32	0.44
		p	0.01	0.32	0.42	0.01	0.09	0.13	0.39	0.16	0.01	0.58	0.11	0.03
Bodily Pain/ Discomfort	26	*rs*	0.06	0.05	-0.05	0.24	0.24	0.04	0.31	-0.01	0.25	-0.14	0.07	0.15
		p	0.77	0.82	0.81	0.24	0.23	0.83	0.13	0.95	0.21	0.50	0.74	0.47
Behaviour	26	*rs*	0.60	0.23	0.04	0.18	0.06	0.39	0.20	0.30	0.51	-0.18	0.37	0.19
		p	0.001	0.27	0.85	0.37	0.78	0.05	0.32	0.13	0.01	0.37	0.06	0.35
Global Behaviour Item	26	*rs*	0.22	0.20	-0.23	0.20	0.31	0.50	0.20	-0.04	0.40	-0.05	0.19	0.29
		p	0.27	0.34	0.25	0.33	0.12	0.01	0.34	0.86	0.04	0.83	0.36	0.15
Mental Health	26	*rs*	0.41	0.17	0.16	0.27	0.25	0.36	0.35	0.33	0.30	0.04	0.29	0.38
		p	0.04	0.40	0.43	0.18	0.21	0.07	0.08	0.10	0.14	0.85	0.16	0.06
Self-Esteem	27	*rs*	0.76	0.31	0.22	0.26	0.25	0.56	0.25	0.29	0.47	-0.06	0.60	0.26
		p	<0.001	0.12	0.27	0.19	0.20	0.002	0.21	0.14	0.01	0.77	0.001	0.19
General Health Perceptions	26	*rs*	0.53	0.40	0.43	0.34	0.41	0.45	0.33	0.42	0.31	0.12	0.60	0.45
		p	0.01	0.04	0.03	0.09	0.04	0.02	0.09	0.03	0.12	0.56	0.003	0.02

: Correlations between CHQ-PF50 and CHQ-CF87. Correlations on scales of the same name are bordered

CHQ-CF87	n		CHQ-PF50 Physical	Role Limitations: Physical	Global Health	Bodily Pain	Role Limits: Emotional/ Behavioural	Time	Parent Emotion	Self-Esteem	Mental Health	Behaviour	PhS	PsS
Change in Health	26	*rs*	0.16	-0.23	-0.31	-0.03	0.16	-0.03	-0.13	-0.36	0.13	-0.04	-0.09	0.09
		p	0.44	0.26	0.13	0.87	0.44	0.88	0.51	0.07	0.52	0.83	0.66	0.66
Family Activities	26	*rs*	0.62	0.40	0.21	0.54	0.53	0.42	0.41	0.40	0.62	0.15	0.57	0.58
		p	0.001	0.04	0.30	0.004	0.01	0.03	0.04	0.04	0.001	0.45	0.002	0.002
Family Cohesion	25	*rs*	0.40	0.20	0.27	0.42	0.43	0.53	0.29	0.20	0.19	0.14	0.47	0.32
		p	0.05	0.34	0.19	0.04	0.03	0.01	0.15	0.34	0.37	0.50	0.02	0.11

p ≤0.01 p≤0.05

5.5.2.2.9. Correlations between the PedsQL and CHQ-CF87

Correlations between the PedsQL and CHQ were performed to determine if the questionnaires measured the same domains and which may be the most appropriate questionnaire for measuring the HRQL of the study group. The correlations of particular interest between the PedsQL self-report and CHQ-CF87 were identified using Spearman' Rank order correlations and include:

- PedsQL Physical: CHQ-CF87 Physical Functioning (rs 0.858, p=<0.001)
- PedsQL Physical : CHQ-CF87 Role Limits- Physical (rs 0.475, p=0.014)
- PedsQL Emotional: CHQ-CF87 Role limits- Emotional (rs 4.12, p=0.037)
- PedsQL Emotional: CHQ Mental Health (rs 0.418, p=0.034)

There were also significant correlations between the CHQ-CF87 Physical Functioning and all PedsQL scales. The full analysis is provided in appendix table 5.20.

5.5.2.2.10. Correlations between the PedsQL parent report and CHQ-PF50

The correlations of particular interest between the PedsQL parent report and CHQ-PF50 were highlighted using Spearman' Rank order correlations and include:

- PedsQL Physical: CHQ-PF50 Physical Functioning (rs 0.687, p=<0.001)
- PedsQL Physical : CHQ-CF50 Role Limits- Physical (rs 0.419, p=0.002)
- PedsQL Physical : CHQ-PF50 PhS (rs 0.719, p=<0.001)
- PedsQL Psychosocial : CHQ-PF50 PsS (rs 0.296, p=0.030)
- PedsQL Emotional: CHQ-PF50 Role Limits- Emotional / Behavioural (rs 0.538, p=< 0.001)
- PedsQL Emotional: CHQ-PF50 Mental Health (rs o.490, p=<0.001)
- There were numerous significant correlations, including CHQ-PF50 Physical Functioning, Role Limitations- Physical, Bodily pain, Parent Time, Mental Health and the PhS composite, and all PedsQL scales. The full analysis is provided in appendix table 5.21.

- There were many often highly statistically significant correlations between corresponding form of the PedsQL and the CHQ, particularly on parent report. In this context, there was evidence of significant relationships between conceptually related scales.

5.5.3. Piers-Harris 2 Children's Self-Concept Scale – Second Editions (PH2)

Self-concept was measured using Piers-Harris 2 Children's Self-Concept Scale – Second Editions (PH2) (Piers and Herzberg, 2002). The PH2 is a self-report questionnaire for children and young people aged 7 to 18 years. Scales include a Total, and six domain scales, namely Behavioural Adjustment (BEH), Intellectual and Social Status (INT), Physical Appearance and Attributes (PHY), Freedom from Anxiety (FRE), Popularity (POP) and Happiness and Satisfaction (HAP). A higher score indicates higher self-esteem or self-regard.

Although self- concept describes an individual's understanding of him / herself and self-esteem describes what one values about oneself, the two are often used interchangeably. For the purposes of this thesis, the term self-esteem will be used.

5.5.3.1. Comparison of PH2 to normative data

The obtained PH2 domain and Total scores were compared to normative values for central tendency of t (by definition 50), as an estimate of the median for non-parametric comparisons with domain scales, and the mean for parametric comparison with the Total. The data on all of the domain scales violated assumptions of normality, and all but one showed some degree of positive skew (appendix table 5.22).

As for previous measures of child psychosocial functioning (PedsQL, CHQs), supplementary parametric analyses of the domain scores are reported in appendix table 5.23. All comparisons indicated that PH2 scores were significantly lower for the LSL group than the normative group.

5.5.3.2. PH2 and gender.

There were no statistically significant effects of gender on the PH2. The results are shown in appendix table 5.24.

5.5.3.3. PH2 and LSL type

Figure 5.12 shows the median PH2 scale scores for each LSL type.

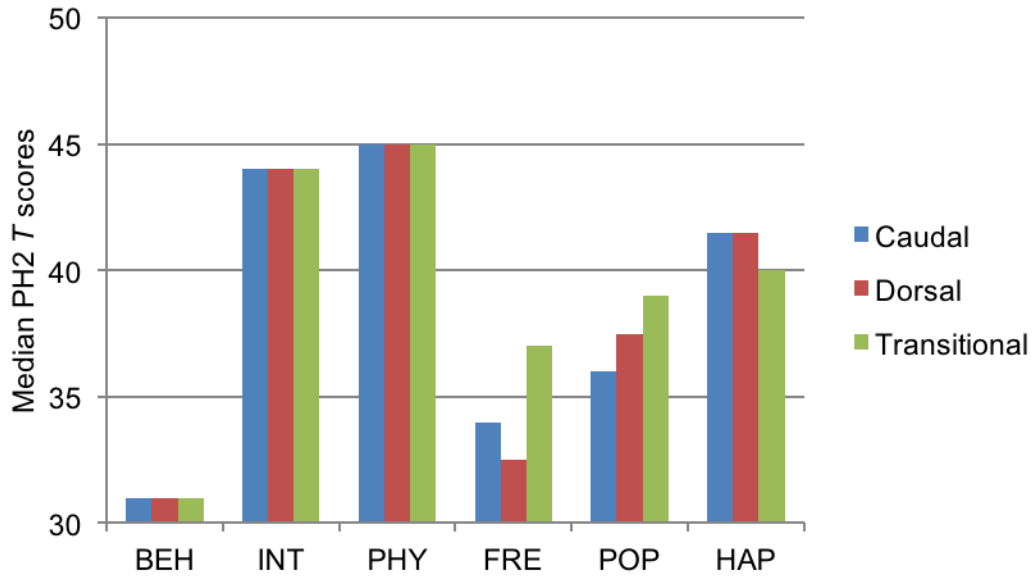


Figure 5.12 Median PH2 scores by LSL type.
The x axis displays domains of the PH2, the y axis the median t scores. The figure shows identical medians for three of the scales (Behavioural Adjustment; Intellectual & Social Status; Physical Appearance & Attributes).

BEH: Behavioural Adjustment; INT: Intellectual and Social Status; PHY: Physical Appearance and Attributes; FRE: Freedom from Anxiety; POP: Popularity: HAP: Happiness and Satisfaction. A higher score indicates a more positive self-evaluation.

The Kruskal-Wallis Test indicated that there were no statistically significant differences between LSL types on PH2 domain scales and a one-way ANOVA indicated that the groups did not differ significantly in their Totals. The results are shown in appendix table 5.25.

5.5.3.4. PH2 and NEM ratings

The relationship between PH2 and NEM variables was evaluated using Spearman's Rank Order Correlations and the results are provided in appendix table 5.26. Only the PH2 Total and NEM Urology correlated significantly (r= -0.39, p=0.01). The correlations between the PH2 Total and NEM Sensory approached significance (r=-0.26, p=0.09), as did the correlation between PH2 Popularity and NEM Bowels (r=0.27p=-0.08)

5.5.3.5. PH2 and Physical Activity

Spearman's Rank Order correlations between ratings on the PH2 and child and adolescent forms of the PAQ (PAQ-C and PAQ-A respectively) are presented in appendix table 5.27. None of the correlations between PAQ ratings and PH2 scales reached statistical significance. Only the relationship between PAQ-A ratings and the PH2 Total approached significance (r=-0.38, p=0.07)

5.5.3.6. Self Esteem (PH2) and pain

The correlations between the PH2 Total and PedsQL PPQ, and PH2 Total and binary pain scores, showed that no differences were statistically significant; the correlation between the PH2 Total and NEM sensory approached significance (rs -0.26; p=0.09).

5.5.3.7. Summary of PH2

Although not statistically significant, the children reported lower ratings on all scales of the PH2 than the normative sample, suggesting more negative general self-evaluations.

There were no clear differences in specific domains between subgroups of young people with LSL, whether in terms of gender, LSL type, experience of pain, or relationships with other clinical features or level of physical activity.

5.6. Discussion

In order to identify the most important parameters to assess, 2 different HRQL and a self-esteem questionnaire were used. Similar methodology was applied to each questionnaire and child and parent ratings were obtained to ensure both perspectives were captured; the exception was the PH2 which has no parent rating available.

Different questionnaires were used in an attempt to obtain the following:

1. Identify if the questionnaire responses correlated with all assessment tools which had been identified as important (for example pain ratings).
2. Identify if one questionnaire was better than another in capturing specific issues.
3. Identify which, if any, questionnaire was specific and pertinent to LSL and user friendly for the outpatient clinic setting.

One of the challenges in researching a rare condition such as LSL is the lack of information regarding the disease process including the HRQL of the children, the effectiveness of interventions and the potential bias of findings. A systematic review of children with lipomyelomeningocele (a subset of spinal lipoma) did not identify any measures of HRQL used with children and young people (May et al., 2013) despite the recognition that a reduced HRQL can have potentially far-reaching consequences in terms of disease related morbidity, relationships, longer-term care and future employment and independence (Sawin and Bellin, 2010).

Results from the current study indicate that children with LSL have a lower HRQL than the healthy population, particularly with regard to physical functioning, self-esteem and the presence of pain. The trend was for the transitional group to have a lower HRQL across lipoma groups. The results are discussed in more detail below.

5.6.1. Statistically significant verses clinically significant results in HRQL measurements

Statistical significance is not the same as clinical significance when measuring HRQL and while statistical significance within a group perspective can inform health policy and treatment programs, for the individual, small changes in HRQL can be hugely relevant regardless of the wider picture (Guyatt et al., 2002, Varni et al., 2003). Methods of calculating statistical verses clinical significance in psychology research are suggested by Varni et al (Varni et al., 2003); however, clinicians must incorporate results from research data, and the patient's clinical presentation and history, to establish the relevance of results.

5.6.2. Child and parent ratings

In contrast to the majority of parents of healthy children who usually rate their child's HRQL higher than the child themselves (Eiser and Varni, 2013), the PedsQL results in the current study indicated that child report was consistently higher than parent report, with parent rating the child's HRQL below the normative value, as identified in other chronic illness literature (Eiser and Varni, 2013). A significant correlation was found between child and parent PedsQL Psychosocial scores in the current study but not the Physical scores and again this is in contrast to the literature, in which parents often agree with the child on Physical rather than Psychological domains (Eiser and Varni, 2013).

However, a significant correlation was found between child and parent rating using the CHQ-CF87 and CHQ-PF50 respectively on Physical Functioning and approached significance for Global Health. As no other significant correlations were found between the CHQ-CF87 and the CHQ-PF50, this suggests differences between the constructs measured and/or between perspectives of the ratings of children and parents, and presents a limitation to using the CHQ.

A lack of agreement between child and parent ratings of HRQL is found in the literature when assessing the HRQL of children with chronic diseases: Law et al (Law et al., 2014) found the majority of proxy ratings were lower than child

ratings in their study of children with physical disabilities, specifically with regard to Physical Functioning, Role Functioning, pain, General Health, Parental Impact and Family Activities; Baca et al (Baca 2010) identified similar findings when using the CHQ to assess the HRQL of children in the community setting and suggested factors affecting this discrepancy included different attitudes, values and understanding between children and adults, and parental concerns regarding their child's longer term difficulties. A further suggestion is that greater discordance between child and parent increases as a disease becomes more active and this is an important factor when evaluating the effect a disease has on HRQL and highlights the importance of longitudinal studies (Vanoni et al., 2016).

Despite the fact that child and parent measurements may provide different results, research suggests that it is important to incorporate both perspectives, in order to inform decision making and assess outcomes (Baca et al., 2010, Eiser and Varni, 2013).

The suggestion is that for LSL, both child and parental ratings should be used.

5.6.3. Gender

PedsQL child report showed females reported a higher HRQL than males on Psychosocial scales and a similar level on the physical scale; PedsQL parent ratings however, indicated that females have a lower HRQL than males on all scales. Burks et al published similar findings, whereby parents rated the HRQL of their female children with asthma to be lower than their male children (Burks et al., 2013). This finding was not replicated in the current study using the CHQ-PF50 where there were no gender differences associated with HRQL. There were no effects of gender on HRQL identified in publications by Varni et al (Varni et al., 2007a) and Lundberg (Lundberg et al., 2012), but Michel et al (Michel et al., 2009) suggests that the HRQL of females decreases with age, but that this is dependent on the measurement scale used.

Higher pain ratings were identified in females using the CHQ-CF87 in the current study and this finding is consistent with the literature (Allen et al., 2009, King et al., 2011). Chronic pain is associated with a reduced HRQL and is discussed more fully in section 5.6.6 HRQL and pain.

The effect of gender of children with LSL requires further investigation to more fully understand the relationship.

5.6.4. LSL type and HRQL

Children with transitional lipomas had a lower HRQL than those with caudal and dorsal lipomas and for most domains, those with dorsal lipomas had the highest HRQL, the exception being that the PedsQL parent rating suggested the caudal group had the highest HRQL. The findings correlated with the clinical results described in previous chapters, whereby the children with transitional LSL had a higher risk of clinical abnormalities and pain, and the dorsal group and to a lesser degree, the caudal group, had the least risk of clinical abnormalities and pain.

There was a trend for the transitional group to have lower scores on the Physical but not the Psychological composite scale using the CHQ -PF50 parent ratings. Child ratings using the CHQ-CF87 also showed the transitional group tended to have lower scores on most measures, particularly with regard to the Family Activity Score, perhaps reflecting the restrictions placed on the family by this group of children in terms of reduced mobility, increased pain and increased urological deficits, including the need to undertake intermittent catheterisation (CIC). A similarity was identified between dorsal and transitional LSL ratings for Parent Time, but there are no clinical findings to explain why the dorsal group may require increased Parent Time compared to the caudal group. The dorsal group scored the highest in terms of Behaviour, Mental Health, and Self-Esteem as identified from the CHQ-CF87 scores and this correlates with reduced clinical deficits and pain, when compared to the transitional and caudal group.

The literature suggests that the greater the child's physical needs, the greater the effect and burden to the child and parent. A study of the HRQL of children with juvenile arthritis suggests that different spheres of the child and parents' lives are affected by the child's disease and present disease specific burdens (Moorthy et al., 2010). This is reiterated in a literature review of children with spinabifida and their families, in which multiple areas are identified as important in relation to the child's HRQL, and may differ between child and parent (Rofail et al., 2013). The authors suggest the child may be more interested in social functioning, activities of daily living and managing pain, whereas the parent may identify parental responsibilities, mental health and work impact as important.

The relationship between specific LSL type and HRQL has not been explored before, but the burden of increased caregiver status is recognised in the literature as being detrimental to the HRQL of the child and healthy family functioning. A study investigating which factors caused the most stress to mothers of children with spinabifida identified CIC as a major cause of stress as it was time consuming for both parent and child, and sometimes painful for the child (Kanaheswari et al., 2011).

Specific clinical variables may affect the child's HRQL and are discussed below.

5.6.5. HRQL and clinical variables

5.6.5.1. HRQL and urology

The current study results show that managing abnormal urology from the child's perspective correlates significantly with Mental Health and Self Esteem, but with no other domains. In contrast, parent ratings show that Social Functioning, Bodily Pain, Self-Esteem and Mental Health all correlate with managing abnormal urinary function.

Bladder and bowel sphincter management are often discussed together in the literature. Inconsistent findings have been identified between continence and HRQL in children and young people with spinabifida, with some authors

suggesting there is no significant relationship between bowel and bladder incontinence and HRQL (Sawin et al., 2002, Lemelle et al., 2006), whilst other studies observe children with spinabifida to have poor scores in relation to bladder and bowel continence and HRQL. Sawin and Bellin (Sawin and Bellin, 2010) reviewed 41 papers exploring the HRQL of children with spinabifida and suggested the inconsistency of findings identified may be due to lack of precision of the instruments used, which have minimal continence domains by which to capture the impact of continence on schooling and socialising. They suggest the need for a HRQL instrument to include these domains to capture their impact more fully on the child's HRQL.

Regardless of the cause, managing faecal / urinary incontinence alongside normal activities at school such as the need for appropriate facilities, privacy and hygiene can be challenging. Social stigma can be attached to incontinence and management of neuropathic bladder and bowel, and can increase difficulties around self-acceptance and disclosure of the problem (Malm-Buatsi et al., 2015). Rendeli et al (Rendeli et al., 2005) studied the HRQL of 29 children and adolescents with spinabifida using the CHQ-PF50; they concluded that parents felt incontinence was a more important factor in the deterioration of the HRQL of their children than deambulation; in contrast, they found parents of the younger children felt ambulation was more important that continence. The authors identified that 52% of their study group felt Mental Health was important in managing urinary incontinence, and both children and parent ratings identified this theme in the current research. Bower (Bower, 2008) identified from presentations at an International Children's Continence Society conference, those children with urological abnormalities transitioning into adult services required understanding and encouragement in managing self-care and promoting mental well-being in adulthood. Environmental and financial factors were also deemed important in promoting mental well-being, with a significant economic burden associated with managing continence (Tapia et al., 2013), although this finding was not examined in the current study.

Parent ratings showed Bodily Pain as important in relation to urology and this is perhaps related to invasive investigations and parents undertaking procedures

(for example CIC), which the children themselves may see as less important in terms of pain. The development of a tool by which to capture the impact of urinary management including CIC as suggested by Sawin and Bellin (Sawin and Bellin, 2010), would be beneficial in exploring this theme further to enable the provision of specific support.

Parent ratings showed Social Functioning as important with regards to urine management, which the children did not identify. There may be many reasons for this discrepancy including the child's age, ability to self-care, schooling status, and confidence. Eiser and Varni (Eiser and Varni, 2013) suggest that discrepancies between child and proxy ratings should not be regarded as "methodological error" but that the views of both may be based on their own experiences and information and as such, both views should be considered. The format and wording of questionnaires may also have an impact and provide a reason for the discrepancies identified above.

The findings suggest that a more sensitive tool may be appropriate for assessing the study group to ensure that the impact that bladder dysfunction has on HRQL is fully understood and addressed.

5.6.5.2. HRQL and bowel function

Deficits in bowel function, including incontinence and constipation, had little effect on the self-rating HRQL of the children in the current study, with reduced Social Scores the only domain identified by the children. In contrast, parent ratings showed deficits in bowel function were associated with reduced Self Esteem, and to a lesser extent, with Role Limitations (Emotional and Behavioural) and Bodily Pain. Kaugars et al (Kaugars et al., 2010) suggests parent and child views may differ regarding the effects of bowel management on the child's HRQL and are partly dependent on family acceptance and functioning, and that this discrepancy can affect the success of treatment. There were no other domains correlated with bowel function in the current study, which again may suggest the lack of precision of the questionnaires used, as

research has identified that abnormal bowel function has an adverse effect on HRQL (Sawin and Bellin, 2010).

The literature suggests that faecal incontinence and constipation can have a negative impact on HRQL, with some improvement in HRQL correlating with improved continence. Ok and Kurzrock (Ok and Kurzrock, 2011) found that 23 children with faecal incontinence rated their HRQL as improved following formation of an ACE (a catheterisable stoma formed from the colon or cecum). The children and parents felt more confident and less anxious, through improvement in the child's continence. The literature review of children with spinabifida undertaken by Sawin and Bellin (Sawin and Bellin, 2010) suggests the difficulty in separating specific symptoms in children with complex needs remains challenging, and presents obstacles in addressing specific needs. Abnormalities in bladder and bowel function are usually assessed together and the current study provides the first attempt to understand the affect that urine and bowel dysfunction as individual entities have on children with LSL. There were 37 children (68.5%) in the current study who were either incontinent of urine or undertaking CIC and 24 (45%) children with deficits in bowel function. Only 5 children (9.2%) had deficits in both urinary and bowel function, thus highlighting the importance of assessing the effect of them separately. von Gontard et al (von Gontard et al., 2011) suggest there is high co morbidity associated with abnormal urinary and bowel function rate in terms of HRQL and that screening for psychological symptoms using specific validated questionnaires is required.

Our data suggests that more research may be required to describe the disparity between our findings and the literature

5.6.5.3. HRQL and physical functioning

In comparison to the general population, children with LSL scored significantly lower in physical functioning in the majority of the measures used. Parent ratings identified that children differed from the normative group in terms of their physical but less so in their psychosocial health.

Parent and child ratings suggest that difficulties related to motor function correlate with a decreased HRQL and these findings are consistent with those from publications relating to children with physical disabilities, where reduced physical ability correlates with a lower HRQL (Law et al., 2014, Sawin and Bellin, 2010). The adolescents within the current group as rated on the PAQ-A, reported that a limited level of mobility as a result of their disease impacts negatively on Family Activities and Family Cohesion, but also on overall Global Health and Perception, Mental Health and Behaviour. Alriksson-Schmidt et al (Alriksson-Schmidt et al., 2007) suggest that adolescents with mobility limitations are at an increased risk of a low HRQL due to many factors including pain, social isolation, hospital visits and missed schooling, and that this needs to be addressed to reduce the impact of associated morbidity as the adolescent transitions into adulthood.

Parent ratings in the current study identified that the child's illness caused limitations to the child participating in physical activities in school and at home. The presence of limitations in physical functioning is recognised as having a negative effect on the family, with environmental barriers including school, resulting in increased difficulties for the child and family (Law et al., 2014). Increased Parent Time correlated with the child's mobility in the current study and this could be explained by the child's young age and mobility status including the use of calipers and other walking aids.

The impact that functional impairment may have on the child's life in terms of friends, school, sport and inclusion in social life is important both at the present time and in terms of resulting long-term morbidity. Maximising functional independence should be a priority for children with LSL and addressing environmental barriers in addition to the child's individual needs are important in providing a holistic approach. The tool provided by the International Classification of Functioning, Disability and Health, Child and Young person Version (ICF-CY), begins to provide such an approach and may be considered in the future for assessing children with LSL (World Health Organisation, 2007).

Chapter 4 highlights the fact that the majority of children (72.2%) have normal neurology, but the results from the current chapter identify that for those who have reduced functional ability, HRQL is affected. Motor function / neurology is therefore an important factor to include in the final assessment tool.

5.6.5.4. HRQL and pain

A publication by The International Association for the Study of Pain (IASP)(International Association for the Study of Pain, 1995) identified a lack of medical training with regard to understanding a child's pain or the impact of parental beliefs and experiences, and that there was a lack of policies and protocols by which to standardise assessment and management of pain in children. The systematic review in chapter 2 identified minimal description or management of pain in children with LSL and no discussion between the relationship between pain and HRQL, thus reinforcing the statement made by the IASP. Children in the current study reported significantly more pain than healthy children, with pain present in half the children, but with very few reporting high levels of pain. Pain was associated with reduced HRQL in all domains on both child and parent report on the PedsQL scale, with a decreased HRQL correlating with increased pain. This finding is consistent with the literature. A study by Wood et al (Wood et al., 2009a) identified that chronic pain had a substantial negative impact on the physical and psychosocial HRQL of children with spinabifida, with chronic pain often being under recognised and under reported by parents and health care professionals (Clancy et al., 2005, Wood et al., 2009a).

There is a correlation between child and parent PedsQL school ratings and pain, suggesting that pain has a direct impact on the child's schooling, or that the activities they undertake at school increases their pain. The literature suggests that in addition to affecting the child's HRQL, chronic pain can affect the child's normal development, have an impact on schooling, and predispose to pain and disability and increased morbidity in adulthood (Gauntlett-Gilbert and Eccleston, 2007). Parental over protectiveness and pain catastrophising can result in a reduction in the child's school attendance and academic results, thus

highlighting the need to intervene and address parental response to the child's pain, in addition to the child's own response (Caes et al., 2011, Logan et al., 2012).

There was a correlation in the current study between Self Esteem and pain, and between parent rating of the child's Mental Health and pain. Forgeron et al (Forgeron et al., 2010) identified that parents of children with chronic pain reported their children to be isolated with few friends, experiencing peer victimisation and being deemed as less popular than their healthy peer group.

However, results from the current study showed that on the CHQ-CF87 subscale measuring perception of improvement in Behaviour, those children who reported pain reported improved behaviour. The reason for this is unclear, but may reflect positive parental support. Meldrum et al suggest that individuals (children and their parents) have varying levels of resourcefulness that effect their ability to cope with chronic pain and that this resourcefulness needs further investigation (Meldrum et al., 2009).

Results from all questionnaires used in the current study identified that pain was correlated with activity and that particularly in adolescents, increased pain was associated with reduced physical activity as confirmed by results from the PAQ-A. The relevance of this finding is that a reduction in physical activity can result in a reduction in overall health in addition to a reduction in self-esteem (Buffart et al., 2009, Malm-Buatsi et al., 2015). Role Limitations (Physical, in addition to Emotional and Behavioural) correlated with the presence of pain in the current study; Social functioning and Emotion are associated with many different variables including the presence (but not the intensity) of pain according to a study of adolescents with chronic pain (Gauntlett-Gilbert and Eccleston, 2007). The authors suggest that the relationship between pain, physical disability and adaptive functioning is not fully understood, but combined, contribute towards reduced school attendance and social interaction.

There was an association between Family Activities and child pain ratings identified in the current study. Family support, activities and relationships are

recognised as predominant factors affecting adolescent participation in activities as highlighted in a study of youths with spinabifida (Vermaes et al., 2007), with the author suggesting these factors influence adolescents' inclusion in both community and school life.

Chronic pain can affect mobility and place further practical and financial burdens on the families of children with chronic pain, thus affecting family functioning and HRQL of the child.

Pain is associated with a reduced HRQL and with over half the cohort of children in the current study reporting pain, it is essential to include a pain assessment in the final assessment tool.

5.6.6. HRQL and social / psychosocial factors

5.6.6.1. HRQL and family factors

The children reported that pain and reduced mobility place limitations on their ability to participate in Family Activities and Cohesion, suggesting that limitations in the child's mobility interrupt family activities and in addition, cause a degree of stress in the family, as identified on the Family Cohesion domain.

The importance of family functioning and cohesiveness in maintaining supportive relationships between parents and siblings and the child with a chronic illness is highlighted in the literature (Alriksson-Schmidt et al., 2007). The authors suggest that supportive and healthy family functioning promotes the child's HRQL and can act as a buffer against adverse events. Future research should focus on the role of the family and evaluation of preventive / supportive interventions in children with LSL which could enhance the child's HRQL.

The effect that LSL has on the child and family has not been examined before and many confounding factors may be indicated, including the age of the child and their individual requirements including medical needs. It is not possible from

the current study to explain these results in depth, but is important for future research to ascertain what psychosocial factors are affected and what interventions may be required.

5.6.6.2. Social / emotional attributes

When compared to the normative population, higher parent ratings in the current study were identified in the area of Global Health, Mental Health and General Behaviour, using the CHQ-CF 50 and this would suggest that many of the children experience good psychological health within the whole cohort.

Parents rate their children as experiencing reduced HRQL due to limitations in mobility, difficulties with urological management and the presence of pain.

Law et al (Law et al., 2014) suggest that higher scores in the psychosocial domain are reported by children with physical disabilities in longitudinal studies, than in the physical domains. The authors suggest that physical disabilities are dependent on the level of physical functioning, environmental barriers, and individual child and family dynamics and behaviour, and that these factors have a direct effect on the child's psychosocial, as well as physical HRQL.

In comparison to the general population, children with LSL also scored themselves significantly lower in Emotional Functioning and were limited in participating in activities due to their emotional difficulties; however, they reported no restrictions in activities as a result of Behavioural Problems. When the CHQ-CF 87 was correlated with the NEM however, results showed increased mobility with increased pain, reduced Global Behaviour with reduced activity level in the adolescent group (PAQ-A), and limitations to participating in activities due to behavioural difficulties. In contrast to the results comparing children with LSL with the normative population, correlating the CHQ-CF87 and NEM scales showed psychosocial difficulties associated with the disease. This again highlights the difficulties in using several assessment questionnaires and suggests limitations to the study as discussed in section 5.7.

5.6.7. Self esteem

Children in the current study had lower levels of self-esteem as measured by the Piers- Harris 2 (PH2) scores in comparison to the general population and there was no association between self-esteem and gender identified. Specific issues relating to reduced self-esteem in the current study are provided below.

5.6.7.1. LSL type and self-esteem

There was no statistical significance between LSL type and self-esteem using the PH2, however there was a trend for children with dorsal lipomas to rate their self-esteem as higher and those with transitional lipomas to rate themselves as having lower self-esteem.

Results from the PH2, although not statistically significant, show a trend for the transitional group to have slightly higher scores in Freedom from Anxiety and Popularity domains and lower in Happiness and Satisfaction domains using the PH2 self-esteem assessment. All three lipoma groups have similar scores for the Behavioural Adjustment, Intellectual & Social Status and Physical Appearance & Attributes domains, with very low scores for Behavioural Adjustment across the group. Since the transitional group tend to have increased clinical deficits and a decreased HRQL, it is unclear why all three lipoma groups have significantly reduced Behavioural Adjustment; this warrants further investigations to identify what the needs and potential obstacles this group of children may perceive as important.

5.6.7.2. Self-esteem and urology

Although there was a statistically significant association between the Total score of the PH2 and impaired urological function, no other domains of the PH2 were associated with low self- esteem. The association between self-esteem and urology is recognised in children with spinabifida: a study by Fischer et al (Malm-Buatsi et al., 2015) identified that children with spinabifida are at high risk of urinary incontinence which is often seen as a social stigma, impacting on

schooling and participation in activities and resulting in reduced self-esteem; those children with improved urinary continence showed improved self-esteem.

A further study examining the influence of urinary incontinence on self-esteem using the PH2 identified that the children's self-esteem improved with enuresis treatment regardless of the success of the outcome; the authors suggest this may reflect the beneficial effect of the treatment process on the child's self esteem (Longstaffe et al., 2000).

A short screening questionnaire has been suggested by von Gontard et al (von Gontard et al., 2011) as a step towards understanding the specific needs of children with urinary incontinence and the authors suggest there is high morbidity associated with this symptom. Such a questionnaire could prove useful for the current study group in providing a deeper understanding of their individual needs.

Urological symptoms would be important to assess in the context of a HRQL assessment. Optimising urological function may well improve LSL patients' self-esteem.

5.6.7.3. Self-esteem and bowel function

Reduced self-esteem and impaired bowel function was identified in the current study and correlated significantly with the Popularity composite of the PH2 scale. This suggests the children with impaired bowel function perceive themselves as being less popular than their peers, are less able to make friends, and feel excluded from activities. Most publications study sphincter dysfunction as a single entity and highlight the difficulty in separating urological and bowel function in analysis: In a study of 57 children with urinary and bowel incontinence who were asked to self-rate the effect that these factors had on their well- being, 57% described reduced self-esteem in association with urinary and bowel incontinence (Bower, 2008). The authors suggest that all self-rating scores are better when bladder and bowel disorders co-exist. The unpredictability of medical symptoms, particularly with regard to bladder and

bowel management can result in lower self-esteem, with episodes of sudden incontinence in a school or social setting, resulting in reduced self-esteem.

An understanding of the children's perception of the impact that dysfunctional sphincter function has on their lives is important in order to inform the effect of treatment and to facilitate appropriate interventions.

5.6.7.4. Self-esteem and physical activity

There was a trend for adolescents to have reduced self-esteem associated with reduced physical activity as measured on the PAQ-A. This result is consistent with findings from a literature review of children with spinabifida undertaken by Lindsay (Lindsay, 2014), who identified that an increased ability of adolescents with spinabifida to manage their physical disabilities had a positive influence on their self-esteem. Increased dependence on others challenges one's self-concept and Lindsay suggests that social competence, improved self-management skills and inclusion in activities, had a positive effect on well-being and self-esteem.

The results reiterate earlier findings in the chapter that suggest functional ability is important to include in the final assessment tool.

5.6.7.5. Self-esteem and physical attributes

There was no association between self-esteem and physical attributes in the study group, despite the fact that the literature suggests there is a link between physical appearance and associated self-esteem, particularly amongst adolescents (Kim-Spoon et al., 2012). The authors suggest that many factors influence self-esteem and physical attributes including age, gender, culture, ethnicity, peer support, parent and media influences. This might suggest that to fully understand the association between self-esteem and physical attributes in the research group, larger numbers of participants are required and potential influencing factors investigated more fully.

5.6.7.6. Self-esteem and pain

PH2 Total and NEM Sensory approached significance (p=0.09), and CHQ-CF87 self-esteem measure and NEM Sensory correlation was significant (p=0.01). The literature highlights the difficulties in assessing the association between pain and self-esteem due to the presence of confounding factors, for example chronic childhood pain can result in school absenteeism affecting peer relationships, academic performance, friendships and reduced self esteem (Esposito et al., 2013). The authors suggest this in turn can aggravate and increase pain perception. Juth et al (Juth et al., 2008) suggest that perceived symptom severity including pain in their study group of children with arthritis related directly with self-esteem and that those patients with low esteem reported increased pain and stiffness.

The association between self-esteem and pain in children with LSL has not been studied before and the research highlights the difficulty in using different assessment methodology; for example, there was no association identified between the CHQ-CF87 domains of self-esteem and Bodily Pain.

5.6.7.7. Summary of self-esteem

Self-esteem predicts HRQL, with low self-esteem resulting in a lower HRQL (Juth et al., 2008). An association between self-esteem and some of the examined variables has been identified in the current study. Children with LSL, many of who already face medical burdens, may benefit from interventions aimed at increasing and promoting self-esteem.

5.7. Reflection on the methodology used

The CHQ, PedsQL and PH2 are generic tools and as such, may be less responsive than disease specific measures. Disease-specific domains may enhance the sensitivity and responsiveness of instruments used, but in the absence of such tools, those best suited for the condition and purpose must be utilised. All the included tools have been validated and offer interpretability.

5.7.1. PedsQL

Parents and children in the current study found the PedsQL simple and quick to complete and minimal time was required for the researcher to analyse. Similar findings were highlighted by Hullmann et al (Hullmann et al., 2011) who examined the attributes and validity of 4 different HRQL questionnaires including the PedsQL and then critically appraised the tool for use in children with rheumatoid arthritis. They found the PedsQL to be an excellent measure of general HRQL with their only criticism being that participants may have difficulty in recalling school related issues if they had been on school holidays and not recently in school.

The generic PedsQL was found to be a good measure of general HRQL and useful in the outcome assessments of hospital based research (Desai et al., 2014). It was therefore considered a useful HRQL measure for children with LSL who are to be assessed in a busy clinical setting.

5.7.2. The CHQ

The CHQ-CF87 and CHQ-PF50 were unacceptable to most respondents in the current study as they found them cumbersome to complete and not adapted for non-American societies due to the vocabulary used and the questions asked. For the researcher, the tools were burdensome to analyse, in addition to which, the CHQ-CF87 and the CHQ-PF 50 do not have consistently comparable domains to enable comparison, as the CHQ-CF 87 does not have summary scores. There was minimal correlation between parent and child ratings using the CHQ-PF50 and the CHQ-CF87, however, although parent report was lower than child report using the PedsQL, correlations between corresponding variables were uniformly significant.

Examination of the degree of association between correspondingly named scales indicated a significant correlation between parent and child ratings of Physical Functioning. The correlation between ratings of Global Health approached significance but all other such relationships were not significant.

This suggests substantial differences between the constructs measured and/or between perspectives of parent and children ratings.

Law et al (Law et al., 2014) suggests that the CHQ was not sensitive to neurological disabilities where subtle changes often occur over a period of time and this presents a limitation for use in children with LSL. A further limitation is presented by Hullmann et al (Hullmann et al., 2011) who identified that both the recall periods required in the CHQ-PF50 (varying across domains from a 4 week recall period, to 1 year, to an unspecified recall time period) and the varying item responses could be confusing to parents. The authors also found that the CHQ-PF50 Total score and the Psychosocial summary score were not responsive in changes in health in their study of patients with rheumatoid arthritis.

5.7.3. The Relationship between the PedsQL and CHQ

There were many, often highly statistically significant correlations between corresponding forms of the PedsQL and the CHQ, particularly on parent reports. In this context, there was evidence of significant relationships between conceptually related scales, particularly with regard to measures related to Physical domains. Although the CHQ-PF50 provides additional information for example the impact of the child's functioning and HRQL on the parent, the findings from the current study highlight some of the difficulties using a tool as complex as the CHQ and suggest the PedsQL is a more appropriate tool for our research group.

5.7.4. The PAQ

Although not a measure of HRQL, results from the PAQ have been used in the current study to provide a measure of activity to correlate with HRQL. The PAQ is simple and quick to complete and analyse, and has been found to be an accurate method of assessing physical activity in an American population (Saint-Maurice et al., 2014). Participants in the current study however found the PAQ inappropriate for describing their activities in the UK (for example, one

question asks how many times they have been ice skating during the past week). The PAQ was therefore considered not to be an appropriate tool for use in future studies of children with LSL in the UK.

Accelerometer- determined physical activity levels have been identified as a useful measure of activity in children and could be a future consideration for measuring the activity levels of children with LSL: Barreira et al (Barreira et al., 2015) found this method of assessment useful in a study undertaken over 12 countries of children aged 9-11 years of age, although it proved challenging to persuade the children to wear the accelerometer over 7 consecutive days for 24 hours per day; a separate study undertaken of 1,494 children in Wales who used an accelerometer and completed the KIDSCREEN HRQL questionnaire (Ravens-Sieberer et al., 2008), found the accelerometer a useful tool by which to assess a child's level of physical activity and their HRQL (Tymms et al., 2016).

The Functional Disability Index (Walker and Greene, 1991) assesses activity limitations in children with pain. The validity of the tool was supported in a study by Claar and Walker (Claar and Walker, 2006) who assessed 596 children with chronic pain and found the Functional Disability Index predicted children's pain and school related disability. This tool could be considered for use in future studies of children with LSL as it encompasses the effect that pain has on limiting activities and includes the school environment.

5.7.5. The PH2

Research has shown children with chronic illness have an increased risk of low self-esteem and that the PH2 has proved a useful method in assessing this domain (Kanaheswari et al., 2011, Gray et al., 2013). The participants in the current research found the PH2 easy and quick to complete and the researcher found it simple to analyse. The PH2 provided a preliminary insight into the self-esteem of children with LSL and could be considered in future longitudinal studies.

5.7.6. Pain assessment

Although not a measure of HRQL, results from the pain assessments have been used in the current study to provide a measure with which to correlate HRQL.

The binary assessment of pain was useful in providing a numerical score to identify any association between HRQL and self-esteem variables, but in a study of 5-18 year olds, was found to be more reliable when accompanied by visual clues, such as valid measurement of pain-related facial expressions (Sikka et al., 2015). The PedsQL PPQ provides a numerical score, a body map for location of pain, and colour coding for pain severity. It provides child and parent ratings, is quick and simple to use and analyse, and would provide a useful adjuvant tool in assessing children of all ages with LSL.

5.7.7. Important HRQL findings in relation to the clinical variables of patients with LSL

Table 5.20 provides details of the HRQL which measure the clinical variables identified as important in relation to HRQL in this chapter.

Table 5.20 Clinical variables important in relation to HRQL as identified in this chapter.

Clinical variables identified as important in relation to HRQL in the study cohort	HRQL measures which assess the clinical variables identified as important in relation to HRQL in the study cohort
Motor function	PedsQL (child and parent versions) CHQ-PF50; CHQ-CF87
Level of physical activity	None
Pain	PedsQL PPQ CHQ—PF50 CHQ-CF87
Urology	None

There were few statistically significant correlations identified between the PH2 and clinical variables and as such, the PH2 was not identified as essential for use in the study cohort.

The correlation between the NEM bowel score and the PH2 ratings approached statistical significance on one domain only, and there was no correlation between NEM bowel function and the CHQ-CF87 ratings or PedsQL self-rating. As such, bowel function was not identified as an essential element of the HRQL measurements for this group of children.

Although measuring the level of physical activity was important in relation to HRQL in the LSL group, the PAQ was not identified as an appropriate assessment tool due to its North American slant and will therefore not be included in the assessment tool. However, a UK version of the PAQ or a

suitable alternative should be considered for inclusion in the final assessment tool.

The effect of urology status on HRQL was not captured in any of the self-rated questionnaires but correlated with Emotion in the parent ratings; as urological function occurs in many children in the study cohort, despite the lack of correlation with HRQL measures urological function was identified as important for inclusion in the final assessment tool.

5.7.8. Summary of HRQL measures

Although the CHQ questionnaires provide data regarding Family Activities, Cohesion and Parent Time involved in caring for children with LSL amongst other domains, the questionnaires were identified as unwieldy to the participants and the researcher in terms of time burden. In addition, the participants disliked the format of the questionnaires and the questions asked.

In contrast, the PedsQL provided a quick and user friendly child and parent measure of physical functioning that was found to be appropriate for the study group.

The PedsQL PPQ provided a quick and user friendly measure of child and parent assessment of pain that was found to be appropriate for the study group.

5.8. Limitations

As with all research the study had some limitations and it is important to address these limitations so they can be overcome in future studies.

5.8.1. Child age

The study group consisted on 54 children aged between 5 and 18 years of age. The questionnaires provided age appropriate questionnaires: the CHQ-CF87 for children between 10 and 18 years of age; the PH2 for 7-18 year olds and the PedsQL, provides questionnaires for children between the ages of 5-7, 8-12,

and 13-18 years of age. The PAQ activity scale provides questionnaires for children aged 8-14 (PAQ-C) and children aged 15-20 (PAQ-A). The LSL types are divided into three groups, transitional (20 children), caudal (19 children) and dorsal (16 children). The study cohort was therefore considered too small to encompass age in the correlations, with the PAC-C for example providing only 3 questionnaires for analysis, thus rendering any statistical analysis meaningless.

5.8.2. Limitations in instruments used

Limitations in the instruments by which to measure HRQL, activity levels and pain have been discussed in section 5.7 Reflection on the methodology used.

5.8.3. Small sample size

A further limitation in the study includes the correlation between small sample sizes of subgroups of LSL and numerous HRQL subscales, which attenuates the statistical power to identify significant differences. For example, the finding of a small number of correlation coefficients in the analysis of HRQL and self-esteem that achieved or approached statistical significance needs to be interpreted cautiously given the relatively high number of tests.

5.8.4. Parametric verses non parametric data analysis.

The choice between non parametric and parametric statistical analysis produces challenges when comparing non parametric study data with normative HRQL data, much of which in parametric. However, non-parametric analysis should be undertaken if the study data does not follow a normal distribution, recognising that with small data sets, the analysis may lack power (McClusky and Lalkhen, 2007).

5.8.5. The child's involvement in research

Children should be involved in research (Modi et al., 2014) and this includes the planning stage where appropriate: Involvement of children with LSL and their parents in future research would enhance such projects by ensuring

researchers are measuring what is important to the child and family. Furthermore, they could help advise on and assist in the development of appropriate support strategies and intervention studies.

5.8.6. Study design

Although cross sectional research cannot be used to look at causality, it proved useful in the current study to address one of the research aims - to evaluate the child's HRQL and to identifying if there is a relationship between lipoma type and HRQL. However, by using cross sectional research, variations in HRQL reports may not have been accounted for; in addition, longitudinal research would have provided data of the individual participant's HRQL as their clinical picture (potentially) changes. This approach should be considered in future studies of children with LSL.

5.8.7. The Economic effect on the family, of caring for a child with LSL

It was not within the remit of this study to consider the financial implications to the family of caring for a child with LSL. However, there are often substantial economic burdens in terms of health costs and productivity losses for the family when caring for a child with a chronic illness, with time from work to care for the child a significant consideration, with a consequent cost to society. The costs to society associated with adolescent chronic pain in America in 2010, was estimated at $19.5 billion annually (Groenewald et al., 2014), with the majority of costs associated with medical costs and loss of family income. The authors suggest that the economic burden for parents caring for a child with chronic pain can affect both the Mental Health and quality of life of both parent and child (Moorthy et al., 2010) and this economic impact alone suggests the need for improved management of chronic pain. This finding raises the question of whether families of children with LSL (with over half the children reporting pain) are facing similar financial burdens and associated stresses.

5.8.8. Summary of limitations

The heterogeneity and variation in symptoms in children with the rare anomaly of LSL make it challenging to assess HRQL and to draw conclusions. Some children may remain asymptomatic whilst other have undergone multiple interventions with associated morbidity, which may affect HRQL. Children may deteriorate over time and therefore the child's age and timing of assessment may be relevant. Finally, there is a lack of other research relating to HRQL in children with LSL, which leaves little with which to compare the current findings.

5.9. Reflection on the challenges

This section will be presented in the first person as it represents a personal reflection.

By addressing the limitations in this chapter, I feel the rigour of the study is improved. As an experienced clinical neurosurgical nurse, I had extensive knowledge of the clinical condition of children with LSL but no experience of qualitative research or statistical analysis. During the research journey I became more familiar with these processes with the support of my supervisors, which gave me more confidence in the analysis. Reflecting on the challenges, I would use fewer methods of HRQL assessment in future research as I feel this confused rather than enhanced some of the outcomes due to the numerous domains studied although given that there was no evidence as to which measures would be most appropriate it was also important to try and identify this for future research and clinical practice. Acknowledging the limitations of the current study, the results add important new knowledge to the HRQL of children with LSL, which has not before been previously explored.

5.10. Conclusion

One of the aims of the thesis is to provide a preliminary analysis identifying if there is a relationship between lipoma type and clinical and HRQL outcomes. The results show that:

1. There is a trend for children with transitional lipomas to have a lower HRQL and the dorsal group to have the highest HRQL, on the majority of measures.
2. Sphincter abnormalities, physical limitations and pain are the clinical variables most associated with a reduced HRQL, and occur most frequently in children with transitional lipomas
3. The results vary according to the tool used, but the results suggest that some of the children have psychological difficulties associated with their disease, and that these needs are not currently being addressed.
4. The results suggest that some of the children face functional limitations that have a negative effect on their HRQL. This would suggest the barriers to their functional participation need to be reduced, with a hopefully consequent improvement in their HRQL.

This information may assist in directing resources to the group most in need of intervention.

One of the challenges in researching a rare condition such as LSL is the lack of information regarding the disease trajectory and the effectiveness of interventions. The World Health Organisation (World Health Organisation, 2001) describes disability from a medical and social perspective. The medical perspective is caused by a health condition and is measured by a professional in terms of functional deficit; the social perspective is an attribute of society, primarily measured by self / parent reports and involves the exclusion of the disabled individual from participation in activities, due to environmental barriers.

Physical disability in childhood and resulting adverse experiences have the potential to lead to depression and poor mental health in adulthood (Austin et al., 2016). A functional impairment scale which measures the extent of restriction in a child's ability to undertake physical, social and personal activities, should be used in conjunction with a HRQL scale. This would provide the researcher with an assessment of the current status of the child in addition to the effect that changes in disease severity have on the child as he / she

matures, and identify what psychological interventions should be implemented to assist the child in coping with such changes over time.

The goal of care for children with a chronic disease and their families is to maximise health and optimise involvement in their environment, whilst minimising morbidity associated with the disease process. Assessing the HRQL of children with LSL can help inform clinical decision making and change the medical paradigm from disease focused, to child and family focused; it can highlight the burden in terms of health and health service use, identify resources required, and aim towards minimising disparities by reducing social and environmental barriers including those within the school environment. This thesis has identified the lack of relevant research regarding the HRQL of children with LSL and presents implications for health and social policy. It has highlighted the requirement for theory driven research by which to identify what factors are important in facilitating holistic care and maximising the child's functioning and participation. Assessment of HRQL with functional impairment data has the potential to guide and inform future research and should be undertaken at national and international level for this rare, chronic anomaly.

This thesis identifies the need for an assessment of HRQL to be incorporated into the overall assessment / monitoring of children with LSL and to form part of the overall management plan.

To ascertain if the Health Related Quality of Life questionnaires addressed the issues the children and parents felt were important in terms of the child's disease, they were asked directly what they felt was important. Chapter 6 describes this process and the findings.

Chapter 6. Understanding what is important to the child with lumbosacral lipoma and their parent.

6.1. Introduction

Few qualitative studies exist that explore the supportive care needs of parents of children with a rare disease, or the supportive needs of the children themselves. It was important to understand in the current study if the generic questionnaires used addressed the issues that were important to the children and parents, and if there were specific topics that they wanted to explain more fully.

A systematic review of the use of generic multidimensional Patient –reported-outcome-measures (PROMs) evaluating meaningful health outcomes for children with disabilities, highlighted the importance of identifying key issues relating to health care that were regarded as important to both the child and parent, in order to address these issues (Morris et al., 2014).

As such issues have not been addressed in the literature for children with lumbosacral lipoma (LSL) it was considered important by the researcher to document any key issues the child and family might identify as important. This data will add to our understanding of this rare anomaly and assist in identifying and addressing inequalities in health care, gaps in the provision of care, and assessing public and environmental health policies that affect this group of children.

6.2. Methodology

After completing their questionnaires, parents and children were asked if there were any issues or concerns that they felt were important in terms of their illness that had not been captured in the questionnaires. The use of interviews was considered, but when participants were asked if they wished to participate in an interview, the majority felt it would be too onerous in terms of time constraints, with children undergoing lengthy and tiring urodynamic assessment

in the morning, followed by an appointment with the neurosurgeon in the afternoon. Most children were in full time education and they / their parents stated they did not wish to miss school on a separate occasion to participate in an interview.

If parents and children did identify issues of importance or concerns (for ease of reading these will be referred to as concerns), they were asked to rank the top five concerns in order of importance, with number 1 being the most and number 5 being the least important. This process was undertaken for each child and parent separately. The researcher entered the answers given by the children and parents into an excel spreadsheet and numerically coded them according to the ranking of importance attributed to them. Once data collection was complete, the answers were grouped together into main themes and another member of the research team independently verified this process. In some instances, parents and/or children provided more detail and this was documented by the researcher and subthemes identified. Child and/or parent descriptions were documented verbatim if this was feasible.

For ease of comprehension, the main themes were then individually colour coded to identify the themes that occurred most frequently. Both the total number of times a specific theme was mentioned and the ranking of each theme were documented, and this process was then repeated for each of the three lipoma types. Differences between issues identified by children and parents and the rank attributed to these concerns were noted.

6.3. Results

The median number of concerns identified by children was 5 (range 0-5) and for parents, the median number was 4 (range 1-5). There were several children who expressed no concerns, thus the lower range for children was 0.

Nine main themes emerged from the child and parent data and these, together with the subthemes, are shown in Figure 6.1.

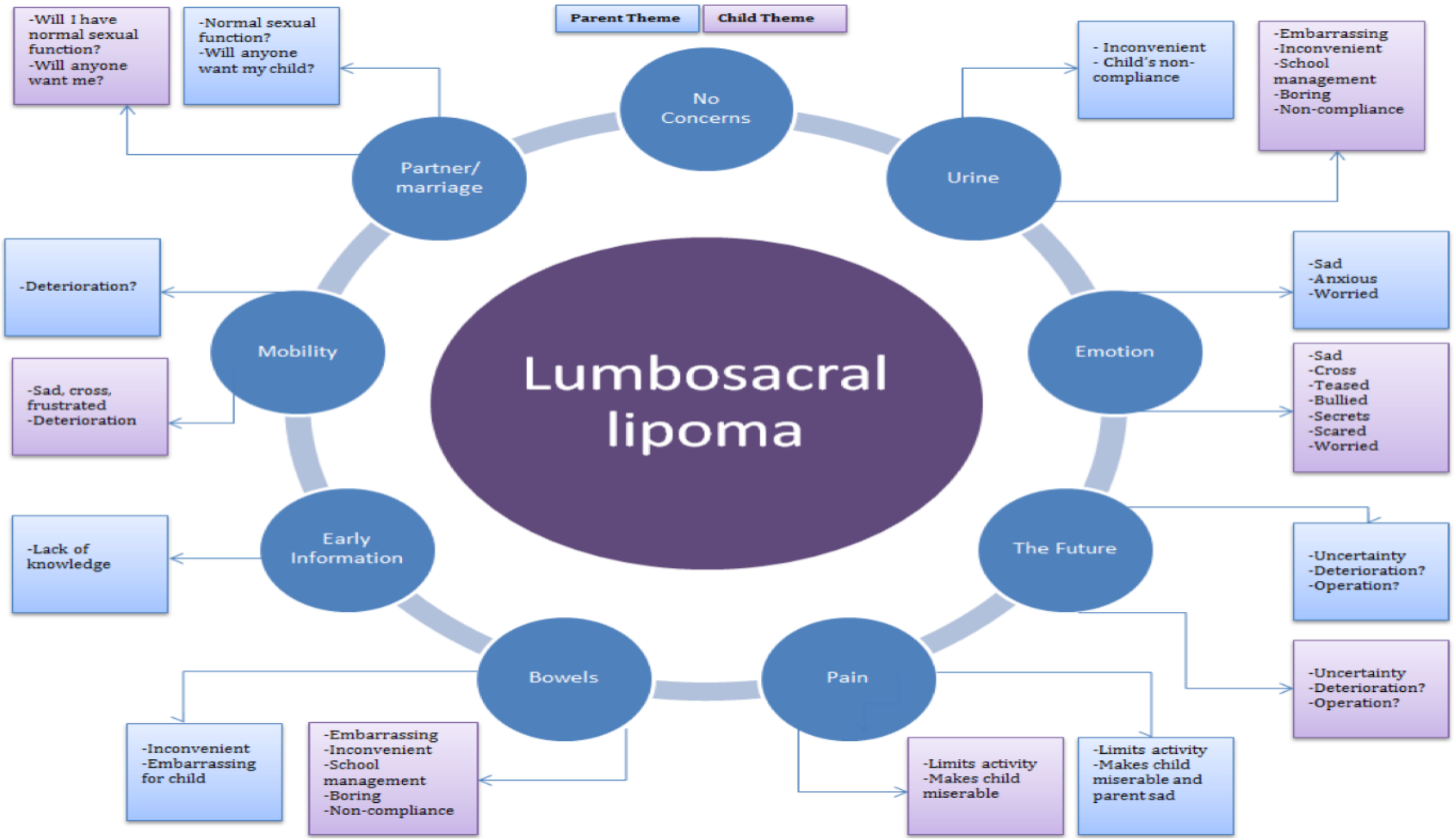


Figure 6.1 The themes and subthemes of importance to children with LSL and their parents

The concerns identified by the child and parent irrespective of ranking are provided in figure 6.2. All parents voiced issues of importance or concerns with regard to the future and many rated this as the most important point to them. Several children said they had no concerns regarding their future. Urine function ranked as important to over 60% of children and was of similar importance to parents.

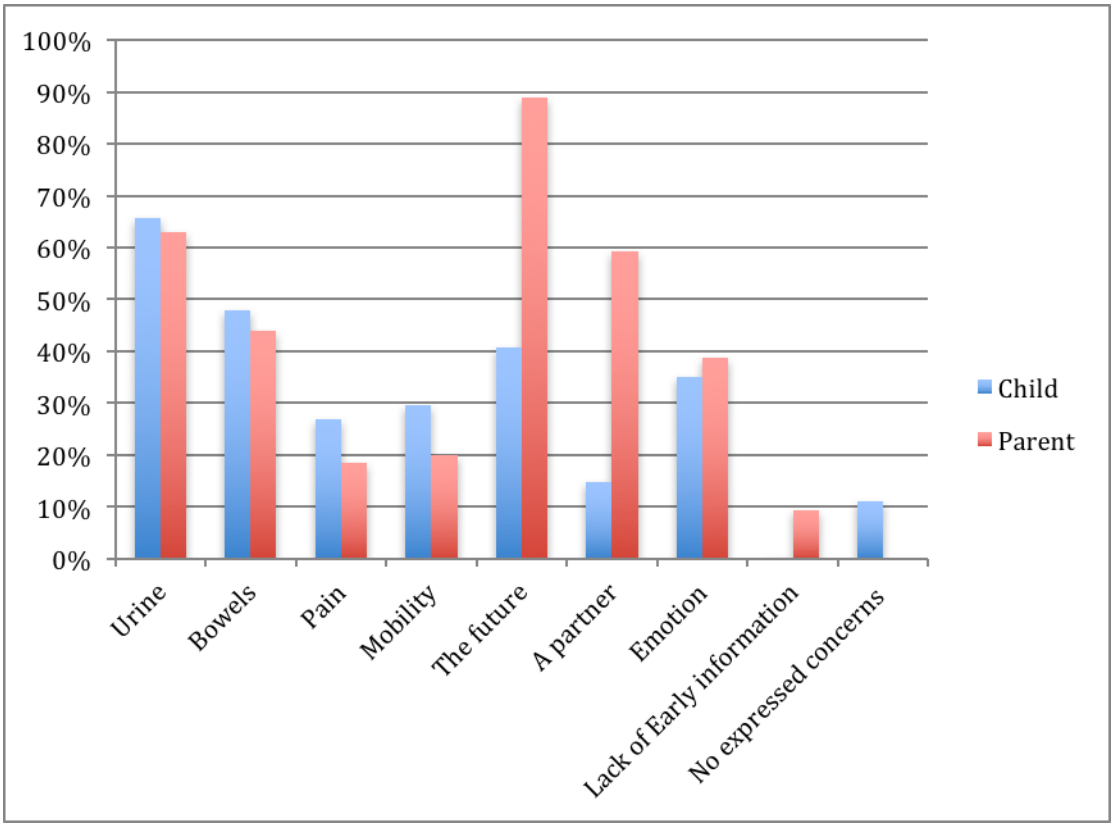


Figure 6.2 The important concerns for children with LSL and their parents
The y axis displays the percentage of the whole group; the x axis the expressed concerns.

The concerns identified as important to children and parents were grouped into themes by the researcher and ranked into order of importance (1 = most important, 5 = least important) and separated into the three lipoma groups as displayed in figure 6.3.

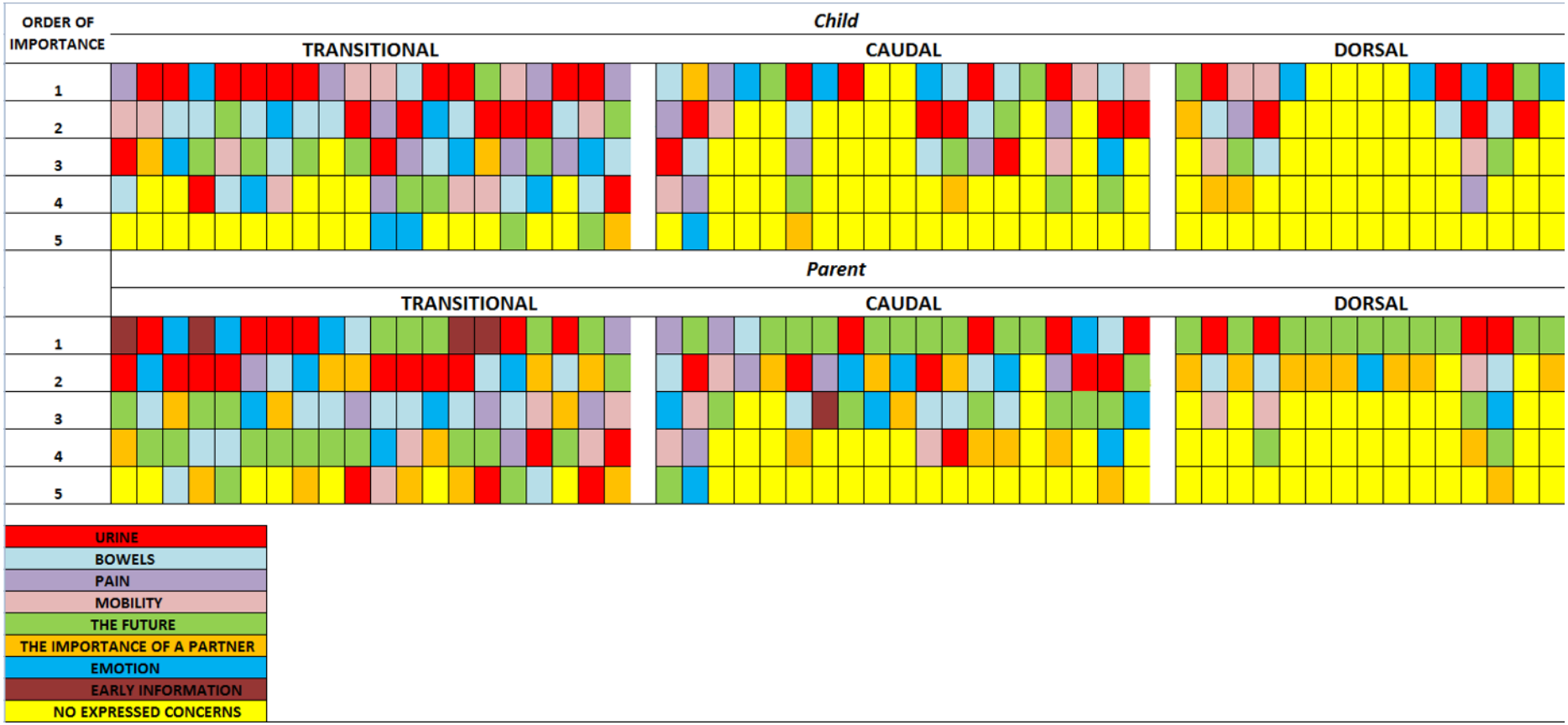


Figure 6.3 Issues of importance to the child with transitional, caudal and dorsal lipomas and their parents

Each of the 9 themes are discussed below:

6.3.1. Urinary deficits

Urinary deficits were described as important in similar numbers of children (68.5%) to parents (62.9%). It was the most important concern to 7 children (31%) and 14 parents (25.9%). There were no children or parents who described it as their least important concern.

Urinary deficits were described as an important concern by all children with transitional lipomas and their parents, with approximately half the group describing it as the most important concern. Children with dorsal lipomas and their parents rated urinary deficits less important than the other lipoma groups. Table 6.1 provides results relating to the three types of lipoma.

Table 6.1 The importance of urinary deficits to parents and children with transitional, caudal and dorsal lipoma

	Transitional n=20		Caudal n=19		Dorsal n=15	
	Child	**Parent**	**Child**	**Parent**	**Child**	**Parent**
N (% of total population)	20 (100%)	20 (100%)	11 (57%)	10 (50%)	6(40%)	4 (26%)
Most important (1)	10 (50%)	6 30%)	4 (21%)	4 (21%)	3 (20%)	4 26%)
Least important (5)	0 (0%)	2 (10%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)

Children identified several concerns that the researcher combined into subthemes relating to urinary management with some describing managing incontinence and intermittent catheterisation (CIC) as “boring”. Children also described the difficulty in managing urinary incontinence in the school environment, where incontinence or the requirement to undertake CIC was

embarrassing and inconvenient. The following quote illustrates a child's embarrassment:

"A boy in year 8 who was also disabled went into my cupboard and found my catheters and spread rumours I use straws to wee. I was really embarrassed as everyone thought it was so funny" (CIC) (12 year old boy with transitional lipoma).

The anger / irritation caused as a result of urinary incontinence is described by an 8 year old boy as follows:

"At school I've got a bag for trousers and pants and all that and then all I need to do when I've wet myself is like we have a special thing for it, there's a file and we need to put our names down when we go toilet so all I need to do is walk out, put my name down, get my bag and go toilet and change. But the other boys tease me and say OOOH he's a baby and has wet himself. I get really cross" (8 year old boy with transitional lipoma).

Concerns identified by parents also included the difficulty for the child in managing urinary continence and this was described as inconvenient, both for the family and the child. Parents described the difficulty in providing sufficient changes of underwear for the child both inside and outside school and in particular for school trips. Some parents were concerned about their child's noncompliance with CIC and the resulting consequences:

"I don't want him to lose his kidneys, last year he didn't do his CIC (Clean Intermittent Catheterisation) and was so ill. He does try, but he is not telling me, he is denying it, I am so worried" (Mother of 16 year old boy with transitional lipoma).

6.3.2. Bowel management

Deficits in bowel function were described as an important concern in similar numbers of children (48%) and parents (44%). It was the most important

concern to 5 (9.2%) children and 3 (5.5%) of parents and the least important to 1 child (1.8%) and 1 parent (1.8%).

Bowel deficits were described as important by more children with transitional lipomas (70%) and parents (75%) compared to those in the caudal and dorsal groups. However, it was children and parents in the caudal group who rated bowel deficits as the most important concern to them (21% and 10.5% respectively). The results are displayed in Table 6.2.

Table 6.2 The importance of bowel deficits to parents and children with transitional, caudal and dorsal lipomas

	Transitional n=20		Caudal n=19		Dorsal n=15	
	Child	**Parent**	**Child**	**Parent**	**Child**	**Parent**
N (% of total population)	14 (70%)	15(75%)	8(43%)	6(31%)	4(26%)	3(20%)
Most important (1)	1(5%)	1(5%)	4(21%)	2(10.5%)	0 (0%)	0 (%)
Least important (5)	1 (5%)	1 (5%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)

Children identified several areas of concern relating to bowel management and, as with urinary problems, some described managing incontinence as “boring”. Managing bowel incontinence was identified as being of particular importance within the school environment, with children describing themselves as being embarrassed of faecal incontinence and the inconvenience associated with this, particularly in the school environment; this is illustrated in the quote below:

“If you have many constant bowel actions you could be having an accident every 20 minutes then either you are going to end up being the product of everyone else’s, you know, the butt of everyone’s’ jokes or you will end up

being very sore, so you know, what's going to happen?" (12 year old boy with caudal lipoma).

The above quote also highlights the difficulty in adhering to bowel regimes in the school setting.

In addition, the necessity of attending to toileting interrupts lessons and disrupts education, and can cause annoyance as highlighted by the quote below:

"Sometimes I get really annoyed cos I have to go to the toilet to clean myself and I come back to the classroom in the middle of something and the teacher asks something and I'm like – what?- cos I missed it, so I just sit there and I'm lost" (9 year old boy with transitional lipoma).

Concerns identified by parents and organised into subthemes by the researcher, included an awareness of the embarrassment caused to their child by faecal incontinence, and the inconvenience for both the child and the family in managing faecal incontinence both inside and outside the school environment. The requirement for frequent changes of underwear and trousers is captured in the quote below:

"He worries how he smells at school if he has soiled himself, he didn't want to wear nappies at school, so he just goes to school with lots of pants, changes in own toilet, it's awful isn't it?" (Mother of 9 year old boy with transitional lipoma).

Some parents described how they needed to be advocates for their children in obtaining appropriate understanding and support in the school setting. In the following quote a mother also identified her awareness that children without such assertive parents may be less fortunate than her own:

"So I think one of the biggest issues for a child who has faecal incontinent issues is getting the right help at school so that they can socialise and they have good self-esteem and confidently enjoy school. Even though she was wearing nappies until she was 7, we had to have constant conversations with

her teacher; there was a person in the classroom with her and that one time was when some really feisty people laughed and pointed at her nappy and she told me that night, I went into see the teacher, she rearranged the whole class and put her with the big softies, problem solved really. Now the thing that worried me about that is we got everything we needed but without 2 parents that were willing to really fight, I think she would have had a horrible schooling" (Mother of 10 year old girl).

6.3.3. Pain

A greater number of children (27%) than parents (18.5%) described pain as an important concern. It was the most important concern to 5 children (9.2%) and 3 parents (5.5%) and no children or parents rated it as their least important concern.

Pain was described as important by more children with transitional lipomas (40%) and a greater number of this group identified it as the most important concern. A similar number of parents in the transitional and caudal groups rated pain as important (26% in both groups) with more parents in the caudal group, rating pain as their most important concern. Children and parents in the dorsal group attributed the least importance to pain as displayed in Table 6.3.

Table 6.3 The importance of pain to children with transitional, caudal and dorsal diplomas and their parents

	Transitional n=20		Caudal n=19		Dorsal n=15	
	Child	**Parent**	**Child**	**Parent**	**Child**	**Parent**
N (% of total population)	8(40%)	5(25%)	5(26%)	5(26%)	2(13%)	0(0%)
Most important (1)	4(20%)	1(5%)	1 (5.2%)	2(10.5%)	0 (0%)	0(%)
Least important (5)	0(0%)	0(0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)

The children described how their pain made them feel miserable and cross. Pain also resulted in limitations in activities both inside and outside the school environment. The following quote illustrates the restrictions perceived by an adolescent girl and her associated frustration:

"I want to walk into town with my friends in high heels like they do, but my legs hurt if I try, so I have to wear really boring trainers" (15 year old girl with caudal lipoma).

Some children used powerful imagery to describe the severity of their pain, as illustrated in the following quote:

"It's like fireworks going off in my legs" (13 year old girl with transitional lipoma).

Parents described the impact that their child's pain has on him / her in terms of participation in activities and on their mood, and the effect this has on the whole family. Parents also expressed their own frustration and sadness at their inability to improve their child's pain, as described by the following quote:

“It feels like his life is ruled by chronic pain and reduced mobility which is very difficult to manage; the whole situation is very unbearable for the whole family and for A. himself. I am at the end of my tether, don’t know how to help him and it’s having a huge impact on his sisters in particular, and that his pain is very difficult to manage on a day to day life. There is very little enjoyment for him, he hopes they might go on holiday but even this doesn’t seem to lift him out of his unhappiness.” (Mother of 10 year old boy with caudal lipoma).

6.3.4. Mobility

A greater number of children (29%) than parents (20%) rated mobility as an important concern. It was the most important concern to 7 children (12%) but to none of the parents.

Mobility was described as an important concern by more children with transitional lipomas (45%) and a greater number of this group identified it as their most important concern. The children who described it as the least important were in the dorsal group.

There was no difference rated by parents across lipoma groups with regard to the importance of their child’s mobility, with none rating it as their most important concern as displayed in Table 6.4.

Table 6.4 The importance of mobility to the child and parent children with transitional, caudal and dorsal lipomas and their parents

	Transitional n=20		Caudal n=19		Dorsal n=15	
	Child	**Parent**	**Child**	**Parent**	**Child**	**Parent**
N (% of total population)	9 (45%)	4 (20%)	3(15.7%)	4 (26%)	4(26%)	3 (20%)
Most important (1)	3(15%)	0(0%)	2 (10.5%)	0(0%)	2(13.3%)	0 (%)
Least important (5)	0(0%)	1 (5%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)

Concerns identified in the current study by children and parents related to the uncertainty regarding potential future deterioration in mobility. A child describes her own concerns as follows:

“So if things get much worse, maybe I will need to use a wheelchair to get around, what do you think?”(15 year old girl with transitional lipoma).

The mother of a young child describes the uncertainty around her child’s future mobility:

“So when these calipers come off, do you think she will be normal, like other kids, or you think she’s going to end up always having a problem with her feet and walking?” (Mother of 5 year old girl with dorsal lipoma)

Some children described that limitations in mobility made them frustrated and irritated as illustrated by the following quote:

“I don’t like these things on my legs!” (5 year old girl with calipers with a dorsal lipoma).

Children described how pain and increasing limitations in activity had a resulting emotional impact, as the following quote illustrates:

“I can do ten minutes, my legs get weak, I used to play football but got pretty self-conscious, had to sit on the side line, I like cricket. One leg’s bigger than other so I struggle even more, when I’m tired I sit out, otherwise the pain gets worse” (16 year old boy with transitional lipoma).

The following quote from a parent describes the effect that restricted mobility has on her daughter and her own awareness of her daughter’s deterioration in mobility:

“She wants to do all the same things as her friends, like walk into town, but it’s all getting more difficult for her now” (Mum of 15 year old girl).

6.3.5. The Future

A greater number of parents (88.8%) than children (40%) identified the future as an important concern. It was the most important concern to 26 parents (48.1%) and 5 children (9.2%) and of the least importance to 3 parents (5.5%) and 2 children (3.7%).

Concerns regarding the future were similar across lipoma groups with a higher percentage of parents in the dorsal group ranking the future as their most important concern as displayed in Table 6.5.

Table 6.5 The importance of the future to children with transitional, caudal and dorsal lipomas and their parents

	Transitional n=20		Caudal n=19		Dorsal n=15	
	Child	**Parent**	**Child**	**Parent**	**Child**	**Parent**
N (% of total population)	11 (20.3%)	18 (90%)	7 (36.8%)	17(89.4%)	4(26%)	13(86.6%)
Most important (1)	1 (5%)	5(25%)	2 (10.5%)	10(52.6%)	2(13.3%)	11(73.3%)
Least important (5)	2(10%)	1 (5%)	0 (0%)	1 (5.2%)	0 (0%)	0 (0%)

There were a number of subthemes identified by the researcher related to uncertainty about the future, one of which was the potential to for the child to deteriorate clinically. One mother talked about her own anxiety:

"I have read about so many stories about it coming back (her symptoms), so yes I worry about that" (mother of 8 year old girl with caudal lipoma).

A further quote from a mother describes how the uncertainty of her child's condition is a constant worry to her:

"I worry and sleep, it's always on your mind, what's going to happen, is he going to be fixed, will he always be like this, what's going to happen at school, how will he manage at school, what will happen when he grows up?" (Mother of 9 year girl with caudal lipoma).

Although parents were informed about the future potential risk of tethered cord with spinal growth, the uncertainty in relation to their individual child can cause anxiety, as illustrated by the following quote:

“I know about tethered cord and that I must look out for pain in her legs and back as she gets older, but I need to know if it’s going to happen to her and when”

The possibility of an operation in the future was also identified, with children and parents being anxious about this possibility:

“Mr T says if my legs get worse I have to have an operation. I don’t want one, I’m scared” (8 year old boy with caudal lipoma)

The mother of the boy quoted above, had similar thoughts regarding potential surgery in the future, as described below:

“I really don’t want him to have surgery, he’s so scared about it, so am I”

Whilst clearly linked to uncertainty regarding the future, some children and parents expressed concerns regarding a future partner.

6.3.6. The importance of a partner

The importance of a partner (boyfriend / girlfriend/ marriage partner) was identified as more important to parents (61.1%) than children (14.8%). No parents identified it as the most important point and 1 child did identify it as the most important point to him / her.

The importance of a partner (boyfriend / girlfriend/ marriage partner) was described as an important concern to more children in the dorsal group, than those in the caudal and transitional group, with 1 child in the caudal group (5.2%) describing it as their most important concern. There were a similar percentage of parents in the transitional and caudal groups who identified the importance of a partner (boyfriend / girlfriend/ marriage partner) as important as displayed in Table 6.6.

Table 6.6 The importance of a partner to children with transitional, caudal and dorsal lipomas and their parents

	Transitional n=20		Caudal n=19		Dorsal n=15	
	Child	**Parent**	**Child**	**Parent**	**Child**	**Parent**
N (% of total population)	2 (10%)	13(65%)	3(15.7%)	9(47.3%)	3 (20%)	10 (66%)
Most important (1)	0(0%)	0(0%)	1 (5.2%)	0(0%)	0(0%)	0(0%)
Least important (5)	1(5%)	7 (36.8%)	1(5.2%)	1(5.2%)	0 (0%)	1(6.6%)

Similar subthemes were identified from the narrative of children and parents and included anxieties around sexual ability and child bearing as described in the quote below:

“Can I have sex like a normal person? What happens if I have kids, I mean, can I?” (17 year old girl with caudal lipoma)

Parents also voiced concerns about sexual function:

“I have talked to him about going out with girls, what you do with them. I worry for him about down below, it is small you know, will a girl accept that, it does worry me. The girl that marries him will be very lucky, but is does concern me. Also, maybe she will think their baby might have the same as him”.

Parents were concerned about their child’s marital prospects both with regard to potential difficulties in sexual ability and pregnancy, but also with regard to the stigma of a congenital abnormality and the potential genetics related to this; finally, parents were anxious that their child would require a partner who would accept them with their specific deficits, and the need as a parent, to feel there

would be someone to care for your child when you yourself were no longer able to. The following quote describes some of these concerns:

"I worry about the future, about whether she will find a nice man to love her and look after her like she deserves. I want her to be happy and to know someone will look after her when I am gone. I worry about her having kids- you know, whether she can, whether they will have problems too" (Mother of 11 year old girl with caudal lipoma).

6.3.7. Emotions

Similar numbers of children (35.1%) and parents (38.8%) identified negative emotions /feelings (worry, stress, sadness, anger) as an important concern. It was identified as the most important issue / concern to 8 children (14.8%) and 4 parents (7.4%).

There were more children in the transitional group who described emotion as important; however, it was rated as the most important concern to children in the dorsal group and of least importance to children in the transitional group.

There were a greater number of parents of children with transitional and caudal lipomas who described emotion as a concern. More parents in the transitional group rated emotion as their most important concern compared to the other groups as displayed in Table 6.7.

Table 6.7 The importance of the emotion to children with transitional, caudal and dorsal lipomas and their parents

	Transitional n=20		Caudal n=19		Dorsal n=15	
	Child	**Parent**	**Child**	**Parent**	**Child**	**Parent**
N (% of total population)	10 (50%)	9 (45%)	5(26.3%)	9 (47.3%)	4 (26.6%)	2 (20%)
Most important (1)	1(5%)	3 (15%)	3(15.7%)	1 (5.2%)	4 (26.6%)	0(0%)
Least important (5)	2 (10%)	0(0%)	1(5.2%)	1(5.2%)	0(0%)	0(0%)

Children described being teased and keeping secrets. The following quote describes how a child was teased about managing his urine:

“I have secrets so I don’t get teased. My good friends know about my wee and stuff, but the big boys tease me about my walking so I don’t want them to know about my wee” (9 year old boy with transitional lipoma).

Some children describe how they want to protect their parents from understanding the degree to which they are being teased:

“I don’t tell my mum everything because she will just get upset” (16 year old girl with caudal lipoma).

An 8 year old describes how he feels his mother is being over protective towards him, which makes him angry. He therefore resists telling her what is happening, as described below:

“If I tell my mum stuff, she will just fuss and fuss and make me cross” (8 year old boy with transitional lipoma).

Some children also expressed anger at their situation as described below:

“It makes me cross. I don’t want to come here and talk about all this with the doctor” (10 year old girl with dorsal lipoma).

Other children described their fear at everything to do with hospital as described by the following quote:

“It makes me scared coming here, all the noises and the smell and stuff” (6 year old girl with dorsal lipoma).

Victimisation and bullying were also identified, illustrated in the following quote: as described below:

“My PE teacher lets me join in with things, but only to a certain extent like last year we were doing cross country run and, he said out loud - Sam I don’t want you to come cos you got troubles”. (9 year old boy with caudal lipoma)

Adolescents in particular recognised the effect their illness had on their family and how that made they themselves feel, as described in the quote below:

“I am really sad that I make my mum and dad so sad” (17 year old boy with caudal lipoma).

Parents also talked about anxiety and fear; the mother of an adolescent boy describes how worried she is that her son may not be compliant with his urine management: .

“Last two years I have worried, every day I worry, when I am at work every day I worry and phone him when he comes home from school, have you done this or that, you know, the urine thing, for two years, it was very scary for us” (Mother of 17 year old boy with transitional lipoma).

Another mother describes the sadness she and her husband feel regarding the effect that their son’s illness is having on their child and themselves:

"It all worries us but we don't let on, but he listens, just have to deal with it, we both cry, he (dad) takes himself into a room and cries but we deal with it. We worry about how it affects him because he is a shy, unpopular boy, eats him up inside" (Mother of 8 year old boy with caudal lipoma).

One parent when asked her views, expressed pleasure at the question:

"It was weird asking me how I feel, cos actually there's quite a lot really I could say and usually no one asks about me, it's always about L. So I quite liked it, you know, someone asking about me". (Mother of 13 year old girl with dorsal lipoma).

6.3.8. The Provision of Information

There were 5 parents (9.2%) who described concerns about the lack of timely information regarding their children's disease and all regarded it as their most important concern. There were no children who described such concerns.

There were a greater number of parents in the transitional group who described their wish for timely information regarding their child's disease and all explained this was the most important concern for them. There was 1 parent in the caudal group who felt early information was important and that this was the most important concern for them. No parents in the dorsal group felt early information was of importance. The results are displayed in Table 6.8.

Table 6.8 The importance of early information

	Transitional n=20		Caudal n=19		Dorsal n=15	
	Child	**Parent**	**Child**	**Parent**	**Child**	**Parent**
N (% of total population)	0(0%)	4(20%)	0(0%)	1(5.2%)	0(0%)	0(0%)
Most important (1)	0(0%)	4(20%)	0(0%)	0(0%)	0(0%)	0(0%)
Least important (5)	0(0%)	0(0%)	0(0%)	0(0%)	0(0%)	0(0%)

Parents describe their concerns at delays in diagnosing their children. One mother explains her frustration at knowing something was wrong with her child and the delay in obtaining a diagnosis:

“It took so long to get to see the right person, she was born with a big lump on her back, all the doctors said it was OK and I was fussing. But I knew something was wrong. She was nearly a year old before I saw Mr. T and it was like…at last, someone believes me” (Mother of 5 year old girl with dorsal caudal lipoma).

Another mother describes how no one was able to explain to her what the matter with her child was:

“If only I understood more when she was little, it was like I was in the dark, no one seemed to be able to tell me what this lump was” (Mother of 10 year old girl with dorsal lipoma).

6.3.9. No expressed concerns

There were 6 children (11.1%) who did not express any concerns regarding their illness and the majority (4) was in the dorsal group (7.4%), with none in the transitional group.

All parents expressed some concerns regarding their child's illness, with parents in the dorsal group having fewer expressed concerns than those in the other groups.

6.4. Discussion

6.4.1. Urology

Both children and parents saw managing urine as an important concern, and the importance attributed to urinary function was similar between children and parents. Children with transitional lipomas and their parents rated urinary deficits as the most important and dorsal as their least important concern. Fischer et al in their study of children with spinabifida, suggest parents and their children differ in their views regarding incontinence (Malm-Buatsi et al., 2015), with parents often not understanding the complexities arising for the child in managing urinary incontinence is the social and school setting. This finding was not however identified in the current study, with children and parents attributing equal importance to managing urinary continence.

The impact that incontinence has on the Health Related Quality of Life (HRQL) of children with spinabifida is poorly researched, with a literature review by Sawin and Bellin identifying a lack of instruments by which to measure this issue (Sawin and Bellin, 2010). Research specific to children with spinabifida involves multiperspective evaluation due to the potential complexity of the condition and the number of clinical deficits involved, making assessment of an isolated symptom difficult. This difficulty applies to the children in the current study although the complexity of their condition does not cover such a broad range of complex health issues as children with more severe types of spinabifida. Urinary incontinence and the amount of inconvenience associated with managing urinary incontinence was identified in the current study, with similar findings evident in the literature: Padua et al highlighted that despite the numerous challenges faced by children with spinabifida, urinary incontinence was an important issue to the children (Padua et al., 2004).

The difficulties associated with managing urinary continence in school were identified in the current study, with similar findings evident in the literature: Schoenmakers et al studied of a mixed group of children with spinabifida and identified the need for sufficient care assistance within the classroom and the difficulties obtaining such care (Schoenmakers et al., 2004). The literature suggests that an increased degree of urinary incontinence is associated with a decrease in social participation (Malm-Buatsi et al., 2015). Further research is required to identify the impact that incontinence may have on children in the current study with regard to the child's social life and participation / restricted participation in activities.

Lack of adherence to treatment regimens is recognised in adolescents with chronic illness (Compas et al., 2012), however noncompliance in managing urinary deficits as highlighted in the current research requires more immediate interventions for the child, to avoid potential renal damage. It is possible that adolescents in particular may be more prone than younger children, to rebel against CIC and the discipline required to undertake this.

The drive to attain urinary continence may place an additional burden on the child and parent. A study by MacNeily et al examined the HRQL of patients with spinabifida before and after lower urinary tract reconstruction, which had been undertaken to improve continence (MacNeily et al., 2009). The authors identified the difficulty in examining only one system in a multisystem disability system, but they did suggest there was an increased burden on patients to attain continence; furthermore, they suggested that imperfect continence and morbidity of treatments results in additional stress and anxiety for patients. Although this was not identified in the current research, it suggests further research would be beneficial for children with LSL.

It is evident that the children and parents in the current study do not feel they receive the support and care they require with regards to managing the child's urinary deficits within their environment. Future plans are to link children with urinary incontinence secondary to LSL with support groups, in order to address the current insufficient care provision. Local health authorities and schools

would also benefit from the resources and educational leaflets provided by support groups.

6.4.2. Bowel management

Deficits in bowel function were seen as important by both children and parents and the importance attributed to this was similar between children and parents. There were more participants in the transitional group who rated deficits in bowel function as important.

It is recognised that faecal incontinence is associated with a reduced HRQL and a study by Wide and colleagues of 172 children with spinabifida identified that children who attained better bowel regimes reported a higher HRQL (Wide et al., 2014). Assessing bowel function as an isolated symptom can be difficult, but similar difficulties arise in the school environment to those attributed to urinary incontinence, with limited resources including insufficient numbers of classroom assistants. The children in the current study identified concerns that are specific to faecal incontinence and soiling, including associated offensive odours.

The effect that bowel management has on children with LSL has not been previously evaluated but has been identified in the current study as important to both the child and family. Taking control of both bladder and bowel continence is a big step towards achieving independence and improving HRQL and has been identified as important in the current study.

6.4.3. Pain

There were a greater number of children and parents in the transitional group who rated pain as important, and more children than parents across all lipoma types, who rated pain as important. The literature suggests that parents may be reliable at reporting only their child's severest pain (Clancy et al., 2005, International Association for the Study of Pain, 2010), and this may in part explain why pain was identified as the most important issue in the current study by 5.5% of parents compared to 9.2% of children. The publication by Clancy et

al also identified that severe pain in children with spinabifida was under-recognised and undertreated by parents and professionals, particularly in the younger children.

Pain as rated by the children must be addressed, as any reduction in the prevalence or severity of chronic pain will benefit the child. Chronic pain is recognised as having a negative impact on the child's HRQL, with low ratings in both physical and psychosocial HRQL scores (Verhoef et al., 2007, Wood et al., 2009b, Young et al., 2013). The literature suggests these factors may also have financial implications for the family, including parental absenteeism from work and financial burden (Groenewald et al., 2014).

Chronic pain in children can lead to functional deficits (Gauntlett-Gilbert and Eccleston, 2007), with musculoskeletal pain in particular leading to reduced mobility and reduced participation in activities (International Association for the Study of Pain, 2010). This restriction in mobility is discussed in the following section:

6.4.4. Mobility

There were more children than parents who rated mobility as important, with the greatest number of children in the transitional group. The literature does not provide an explanation as to why there may be a difference between child and parent rating. The majority of the children in the current study were fully ambulant and it is possible that parents were unaware of subtle changes in mobility that the child might experience, for example moving between classrooms at school. Secondly, some young children in the current study described being frustrated at using orthotics, whilst parents may be able to see the longer-term aim of using such equipment in providing the child with optimum ambulation. In particular, parents and children in the current study voiced concerns about possible future deterioration in mobility as the child grows, with spinal growth being potentially associated with tethered cord syndrome and the consequent requirement for surgery.

Only a small number of children in the current study rated mobility as important, however some children described restricted mobility as making them angry, frustrated and socially excluded. Participation in physical activity within the school environment is an important part of a child's development and the presence of a chronic disease should not pose a barrier to physical activity (Freeman et al., 2013).

The International Classification of Functioning Disability and Health, Child and Youth version (ICF-CY) states that impairment in mobility limits physical activity and can restrict participation in activities, due to barriers within the child's environment. In order to reduce the potential resulting morbidity in adulthood, these issues need to be addressed (World Health Organisation, 2007).

The Equality Act 2010 (previously the Disability Discrimination Act 1995 (DDA)) has made steps towards improving the lives of young disabled people. Within the school setting, The Equality Act 2010 and Schools, suggests that schools must make reasonable adjustment to put disabled children on a similar footing as able bodied children. Further information can be obtained from the website: https://www.gov.uk/guidance/equality-act-2010-guidance.

Little is currently known about the impact that restricted mobility may have on some children with LSL and further understanding and research is required, to enable appropriate resources and funding to be allocated to this group of children.

6.4.5. The future

There were fewer children than parents in the current study that identified their own / their child's future as an important issue. Assessing children's concepts of health including future health can be challenging; young children in particular may be unable to articulate what they feel, have a simplified view of illness, may base their understanding of health and illness on previous experience, and may have a more positive attitude than adolescents (Piko, 2007). Adolescence is a stage of development when behaviour may shape trajectories of disease risk in

the future, with perceived life chances such as education and high expectations for the future, playing an important part in their future health (McDade et al., 2011).

Different research methods may be required to understand the younger child's perception of health and future health and should be considered in future research for children with LSL. An example provided by Piko and Bak describes the draw-and-write technique: 128 primary school children between 8-11years old were asked to describe what they thought about health, illness, prevention and future health; the findings identified that children's drawings in conjunction with writing were an effective method of understanding children's perceptions about health (Piko and Bak, 2006).

A scoping review of 29 studies involving the parents of children with rare diseases identified parents' anxiety over the uncertainty of their child's potential longer term health outcome, with 65% of parents stating the need for more information regarding their child's disease (Pelentsov et al., 2015). The most important issue for some parents in the current research surrounded concerns over their child's future (48.1%). Parental uncertainty about what the future might hold in particular with regard to potential deterioration was paramount.

There were 9.2% of children in the current study who voiced concern over their future. There is a paucity of literature regarding the concerns of children and young people with rare diseases, with regard to their future health. However, in a study of adult survivors of childhood cancer, several concerns were identified by the 28 participants, including marriage, fertility and physical health (NHS England, 2012). The authors highlighted the importance of understanding such concerns in facilitating appropriate support and mitigating these concerns.

In the current study, nearly half the parents and a smaller number of children, expressed concerns about their own/ their child's future. Further research is required to understand these concerns more fully and assist parents and children to acquire and apply coping skills, with which to address their concerns.

6.4.6. A Partner

A small number of children (1.8%) in the current study were concerned about a partner and potential limitation in sexual function, and similar concerns have been identified in studies of young people with chronic diseases. An online focus group discussion with adolescent survivors of cancer involved asking participants about their concerns regarding fertility and potential physical sexual limitations (Nilsson et al., 2014); Knowing that “everything worked” and anxiety about potential genetics of their condition were amongst their concerns, and the authors identified a need for professionals to provide adequate information and support to these young people.

In contrast to the above groups, children with spinabifida have issues specific to sphincter control and this is considered to be a negative factor regarding partnerships and sexual activity. Verhoef et al (Verhoef et al., 2005) investigated the sexual activity of 127 young adults with spinabifida and identified that only 25% had a partner; study participants suggested that incontinence and self-esteem were part of the reason for a lack of a partner.

Dependence on others for activities of daily living in addition to deficits in bladder and bowel control, was recognised as detrimental to sexual activity and finding a partner in young adults with disabilities (Vermaes et al., 2008). The author suggests however that social integration and acceptance is improved if the child is brought up in a mainstream school.

Health care providers often neglect the importance of having a girlfriend or boyfriend, and the sexual limitations and reproductive health of adolescents with chronic disease (Zebrack and Isaacson, 2012). A multidisciplinary team approach should be implemented to address the specific needs of adolescents in the current study group to ensure a healthy transition into adulthood, including counselling in sexuality and partnerships.

Parents in the current study voiced concerns about their child finding a partner in the future to care for them. This concern is identified in the literature and a

review by Fernandes et al highlighted that adolescents with a chronic disease and their parents, voiced similar anxieties regarding the prospect of a future partner and the health of future children (Raat et al., 2002). Further education and support is recommended to address these concerns.

6.4.7. Emotion / feelings

More children than adults in the current study described emotions / feelings as important in terms of their disease. Some described managing their disease as boring and others described themselves as being cross, sad, embarrassed and teased by other children.

Chronic illness in childhood can disrupt a child's normal routine, alter their physical abilities and disrupt their activities and friendships. It can be associated with emotional and behavioural problems in addition to physical symptoms in children with chronic disease, and this is thought to increase the risk of stress for both children and parents in the longer term (Compas et al., 2012).

Being teased as highlighted in the current study, is recognised in the literature: A study of 167 diabetic children identified that teasing, victimisation and bullying were carried out by both teachers and peers, with young children in particular becoming less complaint with their dietary and medicine management as a consequence (Peters et al., 2008). A further study illustrates feelings similar to the current group: Caicedo studied the health of 85 parents of children with chronic disease, who described themselves as feeling anxious, frustrated and isolated (Caicedo, 2014). The author identified the need to recognise these issues and improve the health and wellbeing for the parents and children in their care.

Missing school, changes in and adherence to treatment regimes, feeling different from peers, pain and frustration have all been mentioned by children in the current study. Parents have described similar feelings, along with feelings of sadness. Chronic sorrow has been identified in parents of children with spinabifida (Torres-Ortuno et al., 2014) and the author suggests that separate

assessments of each parent to highlight their individual needs is required , to facilitate the provision of timely interventions.

Further research is required to more fully understand the emotional responses of the children and parents with LSL and to provide appropriate support and intervention, including support within the school environment.

6.4.8. The importance of information

Parents, and to some degree children in the current study identified the need for more prompt information regarding the child's disease and management. Prompt diagnosis and appropriate management of rare conditions is important in ensuring the child receives the correct treatment, and for the parents, to ensure their child does not undergo potentially incorrect procedures and treatment regimes. In a review of 69 children with subaponeurotic fluid collection (DSFC), Worthen et al highlighted the importance of parental awareness and education regarding their child's rare illness, and the benefits of disease specific blogs regarding DSFC (Worthen et al., 2015). The authors suggest that such blogs can increase provider's understanding of DSFC and encourage research, and in addition, increase the provision of appropriate social support for families.

Blogs, the Internet and Support groups provide information and support for families and children. In addition, further education for clinician's outside tertiary referral centres is important for the correct management and referral of children with rare diseases, including those with LSL.

6.4.9. No expressed concerns

Parents in the dorsal group had less concerns than parents in the other lipoma groups and this may be attributed to the fact that this group of children have less clinical deficits, therefore require less frequent investigations and hospital appointments. Children and parents varied across lipoma group regarding the amount of, or lack of concerns regarding their own / their child's illness. A study

undertaken in the outpatient setting of 281 children with serious illnesses and their parents, identified a lack of concordance between parents and young children with regards to the child's distress or concerns; the authors suggest that recognition of the concerns of both the child and parent are required by the clinician to guide appropriate interventions (Penner et al., 2013).

Pelentsov et al (Pelentsov et al., 2015) undertook a literature review of 29 studies examining the parental needs and concerns of caring for a child with a rare, chronic disease. The authors identified multiple needs and concerns including the need for more information (65% of papers), the requirement to address emotional needs (62% of papers) and social needs (72% of papers). Additional parental concerns identified were guilt, uncertainty about the future and addressing their own needs.

Clinicians must understand and address the concerns of children and parents in order to optimise treatment outcomes and ensure the correct provision of social care.

6.5. Summary

Cook suggests that congruence between a child and parent's evaluation of what is important to the child is an essential factor in the subjective measurement of the child's HRQL (Cook, 2003). In her study of children with spinabifida, she identified that children and their parents agreed as to what symptoms / issues were the most important in relation to their disease. Congruence was identified in the current study with regard to urine and bowel management, but less so with other symptoms / issues.

Management of urine function is an important issue for children with LSL and management in the school setting is of particularly importance. Parents rated urine management as important, but slightly less so than the child which suggests parents need to be educated about the difficulties children may face in the school environment. School is a source of socialising and participation in addition to learning, and the individual needs of a child with a disability must be

addressed to enable the child to develop his / her full potential. Education is essential to eradicate teacher and peer victimisation / bullying which is detrimental to the child's wellbeing, and parents and teachers must encourage and promote independence in the child with a disability, to enable the child to gain autonomy and responsibility as a foundation for adult life (Bundy et al., 2015).

Local health and education policies are working to improve the health outcomes for children and young people who have a disability, with the Children and Families Act 2014 seeking to reform the system of support for disabled children from birth to adulthood, across health, social care and education:

(http://www.councilfordisabledchildren.org.uk).

Joint commissioning between health commissioners and local authorities aims to provide medical conditions guidance for schools, in addition to placing the financial budget with the local authority. The aim is to place the child and family at the centre of decision making and care provision.

The surprise and pleasure expressed by one mother in the current study when asked what she thought was important to her and her family, suggests we have a long way to go in understanding and empowering parents in the current study.

Psychology input would benefit many of the children in the current study, to assist compliance with treatment regimes, but also to reduce the risk of associated psychological difficulties continuing into adulthood.

Directing families to the support networks (ERIC, PromoCon, Bladder and Bowel Foundation and SHINE) will be an important step to facilitating support.

Parental distress, anxiety and guilt can occur when the accurate diagnosis of a rare disease is missed or delayed and clinicians must be aware of this clinical entity in order to understand and address parental attitude (Worthen et al., 2015). Parents may refer to the Internet in an attempt to find information and a

review by Schumacher et al suggested a patient / parent -support website may be useful in providing information and emotional support to families (Schumacher et al., 2014). However, LSL can encompass a wide spectrum of clinical deficits with some children being entirely asymptomatic, thus highlighting the need for evidence based published literature.

Pain and mobility are important themes identified, with pain potentially reducing the child's participation in activities and socialising. Future research involving the use of the ICF-CY and the Disability Act is important in integrating this group of children within the school and community environment.

Children do consider what the future may hold in terms of their own health, but with a wide range of ages of participants, it is difficult to extrapolate what their concerns may be in the context of the current study. Some children are thoughtful about finding a boyfriend / girlfriend and what their sexual limitations may be and this is likely to reflect participants in the adolescent group. There are some children who do not have any concerns, these children may be too young to consider their future, or it may reflect the children who are asymptomatic.

Children with transitional lipomas and their parents identified more concerns than those with dorsal lipomas and this correlates with increased clinical deficits in the transitional group as identified in previous chapters. Children with dorsal lipomas and their parents had the least concerns and some children had no concerns at all. Further research is planned to correlate the child's age with symptomology to provide further detail.

The current study identified that parents vary in their perception of their child's needs. This is an important point for clinicians to consider when assessing parents' reports on their child's disease progression and adherence to treatment regimes, particularly in adolescents.

The paucity of reviews regarding issues of importance to children with LSL and their families identifies the need for further research in order to provide appropriate support, interventions and policies.

Concerns for their child's future were the most important concern for parents and this reflects the unknown natural history of the disease and the difficulty associated with this uncertainty. The current research aims to address some of this uncertainty by linking types of lipoma with individual clinical outcomes and thus suggesting potential predictions for children with specific lipoma types. Educating parents and publishing results of the study will enable a wider group of parents' access to research based evidence.

When comparing children in the current study with publications relating to children with spinabifida which encompass the most severe spectrum (myelomeningocele), it is important to consider that the majority of children in the current study have fewer major deficits. The myelomeningocele group also has the potential for cognitive deficits, making the extrapolation of identifying an isolated concern more challenging.

Future research is needed to educate parents and health care professionals about the nature and prevalence of concerns to children with LSL and their parents, and to develop intervention and management programs, including guiding Government policies.

Children and families may find supportive resources helpful including support groups, through the sharing of experiences and challenges, and health care professionals should signpost them to support groups appropriate to their needs (Hema et al., 2009).

The following support groups have been identified as potentially helpful for children with LSL in managing some of the themes and subthemes discussed above.

- ERIC is a support group dedicated to helping children with urinary incontinence and was started by the Children's Society in 1988 (http://www.eric.org.uk/). The group is a member of the Paediatric Continence Forum (PCF) and is assisting commissioners to identify appropriate resources and pathways across schools, NHS care and social services. In addition, ERIC assists health care professionals and schools in understanding and addressing issues relevant to the children.
- PromoCon is a support group for both urinary and faecal incontinence, with education and resource information: (http://www.disabledliving.co.uk/PromoCon)
- The Bladder and bowel foundation provides information and support, including a helpline to people of all ages with incontinence: https://www.bladderandbowelfoundation.org/
- SHINE is a support group for children with spinabifida and hydrocephalus and has an extensive section regarding the management of sphincter dysfunction: (http://www.shinecharity.org.uk)

To further understand the effect that living with a child with LSL has on the family, parents were provided with appropriate questionnaires and the process and results are provided in chapter 7.

Chapter 7. A preliminary analysis of the psychological effect of parenting a child with lumbosacral lipoma

One of the aims of the thesis was to evaluate the impact of lumbosacral lipoma (LSL) on the child and parent. The previous chapters have evaluated the impact on the child and to a lesser degree, the parent. This chapter describes in greater detail, the effect that living with this chronic disease has on parents.

7.1. Introduction

It is important to explore how children and young people perceive their day-to-day living alongside illness, to understand how it impacts on their daily lives. However, the importance of parents can play a major role on the child's health related quality of life (HRQL) and the attitude of children to illness can be very dependent on the behaviour and attitude of their parents (RCPCH, 2013). Although parents can be, and often need to be, strong advocates for their children, they may also become overprotective resulting in conflict as the child grows and becomes more independent (Mullins et al., 2007).

Palermo et al (Palermo et al., 2008) suggest that becoming a parent is a life changing event. The daily demands of parenting, managing the household including caring for children of different ages, maintaining employment and managing finances, can be challenging for parents of healthy children; when faced with a child with a chronic illness, family and social roles are altered, with parents facing the often complex burden of caring for a child with a chronic illness and managing associated stresses. A systematic review involving the qualitative analysis of 96 studies and meta-analysis of 13 studies explored the stress experienced by parents of chronically ill children (Cousino and Hazen, 2013) and identified increased levels of stress, depression and anxiety in parents of children with a chronic disease; increased disruption to family functioning and increased financial burden were also identified.

While a significant source of strength through a close relationship with their parents is important, the demands of childhood chronic illness can place unique

demands on the entire family, and failure of family members to adapt accordingly can be an important risk factor for the development of parental psychopathology (Caicedo, 2014). Parenting is important in the management of the child with a chronic disease, and parental psychological distress has been identified as a risk factor for poorer outcomes in children with a variety of chronic health conditions (Eccleston et al., 2015, Palermo et al., 2014).

It was therefore considered important to explore the psychological functioning of parents of children with LSL in order to understand if they were at risk of increased stress, anxiety or depression, and to consider if interventions were required to promote positive outcomes for both the parent and child.

7.2. The effect on the child

The systematic review by Cousino and Hazen (Cousino and Hazen, 2013) identified that parenting stress, in addition to having a negative effect on the parents themselves, also contributed to poorer child health outcomes, with higher levels of childhood depression and poorer emotional control associated with higher levels of parental stress. The review identified gaps in the literature regarding the relationship between parental stress and child psychological and clinical outcomes and suggested that further research was required to inform future interventions. A separate study investigating the relationship between parental stress and child health outcomes was published by Barakat et al (Barakat et al., 2007). The authors identified that parents of children with newly diagnosed sickle cell disease experienced higher levels of stress in association with lower family functioning, reduced coping abilities, poorer health outcomes and increased health care usage; they suggest that improving family functioning may impact directly on child health outcomes.

7.3. Parenting a child with a chronic disease

There is a paucity of publications regarding the needs of parents of children with a rare disease and none regarding parents of children with LSL. However, a literature review of 29 studies examining parents' needs when caring for a

chronically sick child identified that uncertainty, lack of information, emotional and social needs were the common issues cited by parents as important (Pelentsov et al., 2015). Uncertainty and anxiety regarding their child's potential disease progression have been recognised as a source of stress and depression in parents of children with chronic diseases, with a corresponding reduction in parental HRQL (Mullins et al., 2007). The authors also suggested that there is an association between parental stress and adverse caregiver sequelae, in addition to a negative effect on the child's psychological state. It has also been identified that the level of maternal depression and anxiety increased with the more dependent and disabled child (Al-Eithan et al., 2013).

A systematic review by Vermaes et al (Vermaes et al., 2007) identified that parenting a child with spinabifida had a negative effect on parents' psychological adjustment. The authors highlighted that with such a complex condition it was difficult to extrapolate specific reasons for this negative effect, but that child, parent, family and environmental factors were all associated with parental psychology and adjustment. Maintaining healthy functional relationships with other family members and health care professionals was suggested by the authors as being a positive method by which to manage disease uncertainty, carer burden and addressing the changing needs of the child.

7.4. Methodology.

All 54 parents were asked to complete the Hospital and Depression scale (HADS) and the Pediatric Inventory for Parents (PIP) questionnaires.

The HADS is a self-report questionnaire comprising separate scales for anxiety (HADS-A) and depression (HADS-D) (Zigmond and Snaith, 1983). The 14-item questionnaire is scored on a Likert scale of 0 (not present) to 3 (considerable) with a resulting score of between 0-21 for either depression or anxiety.

The PIP is a 42-item self-report questionnaire, yielding scales for Frequency and Difficulty related to Communication, Medical Care, Role Function,

Emotional Function and a Total score (Streisand et al., 2001). While each item is rated on a 5 point scale, each domain comprises different numbers of items (Communication = 9, Medical Care = 8, Role Function=10, Emotional Function = 15; Total=42). Consequently, scores are not directly comparable.

The methodology is described in chapter 3, along with the reasons for tool selection.

7.5. Results

7.5.1. HADS

Tests of normality indicated that the data were positively skewed (Appendix 7.1) and this was more pronounced for the HADS-D than the HADS-A. Consistent with this, the distribution of the HADS-D scores significantly deviated from normality, whereas analysis of HADS-A scores failed to reach significance ($p=0.06$). Under these circumstances non-parametric methods were used for further analyses.

Bjelland et al. (Bjelland et al., 2002) recommended a cut-off of 8 and above for identification of caseness (the threshold at which it is appropriate to initiate treatment) for both the depression and anxiety scales. Therefore, scores were also analysed in terms of whether they were <8 or 8+.

7.5.1.1. Comparison of HADS to normative data

The obtained HADS scores were compared to normative data from a UK non-clinical sample (Crawford et al, 2001). Crawford et al reported medians of HADS-A=6 (IQR=5) and HADS-D=3 (IQR= 5).

1-sample Wilcoxon tests indicated that the Parents of children with LSL reported significantly higher levels of anxiety ($p<0.001$) but not depression ($p=0.23$).

7.5.1.2. Cut-offs for clinical caseness

Application of Bjelland et al.'s cut-off indicated that 29 out of 54 respondents (53.7%) reported anxiety above the cut-off, whereas 11 (20.4%) reported depression. The proportion of HADS-A cases was significantly higher than HADS-D cases ($p<0.001$).

It was notable that all 11 instances of HADS-D cases were also identified as cases by the HADS-A. That is, there were no cases of depression without anxiety. This indicated a statistically significant association between depression and anxiety ($p= 0.002$).

7.5.1.3. Parental HADS and Child Gender

There were no statistically significant differences between HADS scores and child gender (HADS-A $p=0.73$; HAD-D $p=0.74$ Mann Whitney U test) (appendix 7.2).

7.5.1.4. HADS and the presence of a syrinx

There were no statistically significant differences between HADS scores and the presence / absence of a syrinx in the children with LSL (HADS-A $p=0.22$; HADS -D $p=0.90$ Mann Whitney U test) (appendix 7.3).

7.5.1.5. HADS and LSL type

Figure 7.1 provides the Median HADS scores by LSL type. This shows that the median anxiety score was higher than the median depression score in each of the groups (appendix 7.4).

It also suggests a trend in terms of caudal cases being associated with the lowest levels of parental psychological distress and transitional cases being associated with the highest.

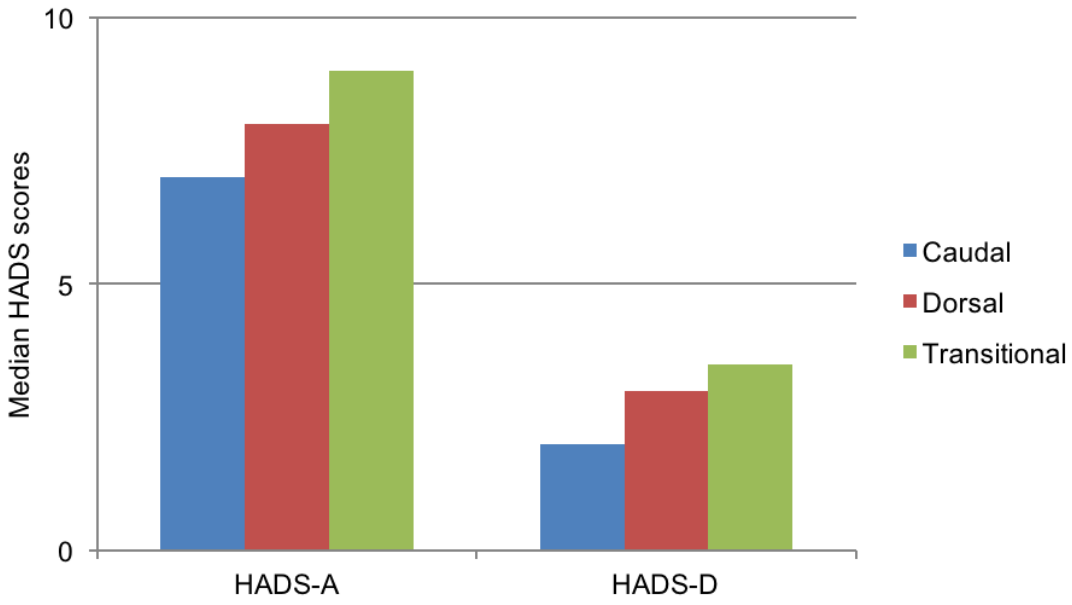


Figure 7.1 Median HADS scores by LSL type

The x axis displays HADS A (anxiety) and the y axis HADS D (depression); the y axis displays the median HADS scores; the results show a trend for the transitional group to have the highest level of anxiety and depression.

7.5.1.6. HADS and NEM ratings

The relationship between HADS and NEM variables is shown in table 7.1

Both anxiety and depression were significantly correlated with NEM Total scores. Furthermore, HADS-D was significantly correlated with NEM Urology.

Table 7.1 HADS and NEM ratings

Table 7.2 HADS and NEM ratings (n=54)										
	NEM ratings									
	Motor		Sensory		Urology		Bowels		NEM Total	
	rs	p	rs	p	rs	p	rs	p	rs	p
HADS-A	-0.12	0.38	-0.10	0.46	-0.21	0.13	-0.08	0.55	0.28	0.04
HADS-D	-0.18	0.19	-0.13	0.36	0.29	0.04	0.03	0.85	0.31	0.02

p ≤0.01 p≤0.05

7.5.1.7. HADS and Pain

The comparisons of HADS ratings in parents of children who did or did not report pain shows a trend towards higher anxiety and depression scores in parents of children in pain, although this finding was not statistically significant. The results are provided in appendix 7.5

7.5.1.8. HADS and Physical Activity

Correlations between ratings on the HADS and the PAQ-C and PAQ-A shows none of the correlations between PAQ ratings and HADS scales approached statistical significance. The results are provided in appendix 7.6.

7.5.1.9. Summary of HADS results

There was a higher anxiety score than depression score identified.

The results indicated the presence of anxiety and depression in parents of children with LSL, with anxiety being more prevalent than depression. There was a trend towards parents of children with transitional lipomas having the highest level of anxiety and depression and the parents of children with caudal lipomas the least. Both anxiety and depression had significant relationships with the NEM Total rating of clinical symptoms.

7.5.2. Pediatric Inventory for Parents (PIP)

Tests of normality indicated that the data were positively skewed and non-parametric was consequently used. The results are provided in appendix 7.7.

7.5.2.1. PIP and child Gender

There were no statistically significant differences identified between PIP scores and child gender (Total Frequency score p=0.96; Total Difficulty score p=0.90 Mann Whitney U test) (see appendix 7.8).

7.5.2.2. PIP and the presence of a syrinx

There were no statistically significant differences identified between PIP scores and the presence / absence of a syrinx in children with LSL (Total Frequency score p=0.50; Total, p=0.43 Mann Whitney U test). (appendix 7.9).

7.5.2.3. PIP and LSL type

Figure 7.2 shows the median PIP scale scores for each LSL type. To simplify presentation on a single Y-axis, they are expressed as a proportion of the maximum for each scale.

This suggests that concerns regarding medical care were both the most frequent and the most difficult.

Although not statistically significant, the results show a consistent trend for the parents of children with transitional lipomas to report higher scores in both the Frequency and Difficulty scores in each of the PIP domains.

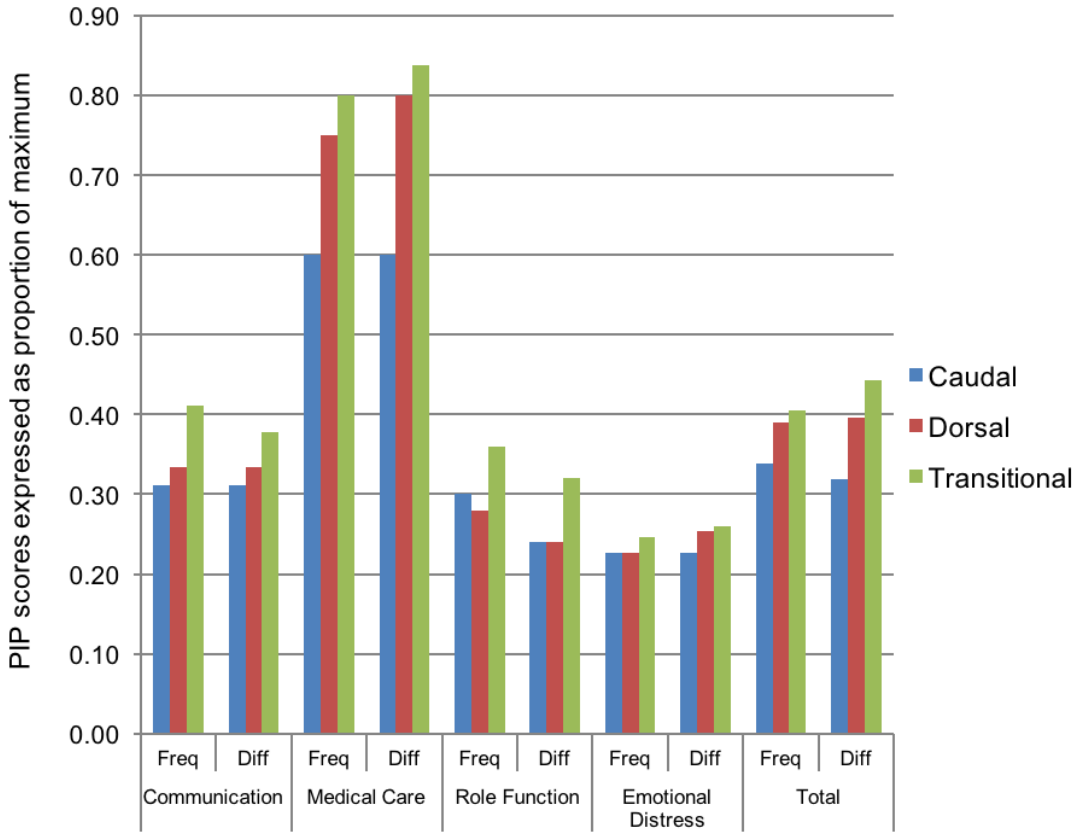


Figure 7.2 Median PIP scale scores for each LSL type

The x axis shows each of the PIP domains; the y axis the PIP scores; the transitional group have higher ratings in all domains.

7.5.2.4. PIP and NEM ratings

The results are provided in table 7.2 and show that NEM Motor and Total ratings were significantly correlated with all PIP scales. Correlations between NEM Urology either were significant or approached significance for all scales except PIP Role Function.

In contrast, NEM Sensory correlated significantly only with PIP Emotional Distress. There were no statistically significant associations between NEM Bowels and the PIP.

Table 7.2 PIP and NEM ratings

NEM Total scores significantly correlated with all PIP scales, as did the majority of NEM Motor scores and many of NEM sensory scores.

PIP and NEM ratings (n=54)											
		NEM ratings									
		Motor		Sensory		Urology		Bowels		NEM Total	
PIP		rs	p	rs	p	rs	p	rs	p	rs	p
Comm.	F	-0.26	0.06	-0.17	0.21	-0.31	0.02	-0.14	0.30	-0.37	0.01
	D	-0.26	0.06	-0.21	0.13	-0.26	0.05	-0.09	0.53	-0.32	0.02
Emotional	F	-0.38	0.01	-0.30	0.03	-0.26	0.06	-0.07	0.60	-0.38	<0.001
	D	-0.34	0.01	-0.29	0.03	-0.29	0.03	-0.11	0.42	-0.41	<0.001
Medical	F	-0.33	0.02	-0.18	0.19	-0.37	0.01	-0.18	0.18	-0.47	<0.001
	D	-0.35	0.01	-0.26	0.06	-0.28	0.04	-0.18	0.19	-0.42	<0.001
Role	F	-0.26	0.05	-0.18	0.20	-0.20	0.14	-0.02	0.91	-0.29	0.03
	D	-0.28	0.04	-0.18	0.20	-0.18	0.19	0.02	0.88	-0.28	0.04
Total	F	-0.34	0.01	-0.22	0.11	-0.30	0.03	-0.11	0.44	-0.41	< 0.001
	D	-0.33	0.01	-0.25	0.07	-0.26	0.06	-0.09	0.51	-0.37	0.01

p ≤0.01 p≤0.05

7.5.2.5. PIP and Pain

PIP scores were consistently higher for parents of children who experienced pain than those who did not. None of the comparisons were statistically significant, although those for Medical Care approached this level (Frequency score p=0.08; Difficulty score p=0.09 Mann Whitney U test). The results are provided in appendix 7.10.

7.5.2.6. PIP and Physical Activity

None of the correlations between PAQ ratings and PIP scales were statistically significant. Only that between PIP Emotional Distress and PAQ-A approached significance (p=0.06 Spearman's correlation). The results are provided in the appendix 7.11.

7.5.2.7. Summary of PIP

Concerns regarding medical care were both the most frequent and the most difficult areas for parents, with a trend for the parents of children with transitional lipomas to report higher scores in the Frequency and Difficulty domains of the PIP Scale and the parents of children with caudal lipomas the least.

Motor, urology and pain correlated significantly with almost all domains of PIP ratings with an increase in these PIP ratings. Higher PIP scores were associated with higher child's pain scores.

7.6. Discussion

7.6.1. Type of LSL

Children with transitional lipomas have a higher number of abnormal clinical symptoms as described in chapter 4. This might account for the higher scores identified in the transitional group in the Frequency and Difficulty domains in each of the PIP ratings, and higher parental anxiety, and to a lesser extent,

depression ratings, in the HADS scores for parents of children in the transitional group. Results from a study by Gray et al (Gray et al., 2013), suggest that parents of children with more severe disease are at an increased risk of greater stress, and this is supported in the findings from the current study.

Medical Care was the specific domain associated with the greatest parental anxiety and depression. The literature supports these findings, with higher levels of parental anxiety and depression corresponding with the increased needs of more physically disabled children and the assistance the children need in everyday life (Sanchez-Lopez et al., 2015); a literature review exploring the parenting of children with spinabifida (Vermaes et al., 2007), identified that not only was an increase in the child's physical disability associated with increased parental stress, but that the fluctuating care needs of the child corresponded with changes in parental stress levels. This suggests that longitudinal evaluation of parental emotion is needed to understand what type of support and when such support may be required.

In the current study, parents of children in the caudal group reported the least anxiety, depression or parental concerns, although results from the clinical chapter identified the dorsal group, rather than the caudal group, as having the least abnormal clinical symptoms. The caudal group had the highest incidence of bowel dysfunction, higher than the dorsal group with regard to urological abnormalities, yet the lowest incidence of neurological abnormality. The reasons behind the discrepancies between increased clinical abnormalities and parental emotion are unclear and suggest the need for further investigation. Cousino and Hazen (Cousino and Hazen, 2013) suggest that greater parental responsibility for treatment management is associated with increased parental stress, rather than illness severity. These findings would suggest that parents of children with transitional lipomas undertake greater responsibility for treatment management, and this theme is explored in the following sections.

7.6.2. Mobility

Neurology and orthopaedic function were analysed separately, although both contribute to mobility and as such are discussed together. The level of activity as measured using the PAQ questionnaire provided details of the child's participation / restriction/ avoidance in daily activities. There were a total of 15 children (27.8%) who had abnormal neurology of whom 5 (9.3%) had deteriorating neurology, and 11(20.4%) had abnormal orthopaedic function of whom 1 (1.8%) was deteriorating.

No significant correlations were identified between parental anxiety and depression domains of the HADS scores and the child's motor scores using the NEM and the PAQ. However, correlations between all domains of the PIP scale and motor scores were identified and between the PIP Emotional domain and PAQ-A scores.

Parents rated communication more difficult and more frequent on the PIP questionnaire, be it with the child, their partner or a health care professional, and this finding correlated with the child's motor function. Parents also demonstrated increased Emotion as rated by the PIP questionnaire, which the authors of this questionnaire suggest is associated with changes in parental sleep pattern and mood (Streisand et al., 2001).

Carrying out their child's medical care requirements and the effect that their child's illness had on paternal ability to care for other family members and undertake work themselves, correlated with the child's motor score. However, the amount of physical activity the child undertook did not correlate with levels of parental anxiety and depression; the only correlation found was between the activity level of the adolescent participants and the parental Emotion domain, which suggests that either adolescents were less mobile than younger children, or that more importance was placed on activity and participation during adolescence, by parents.

When comparing able-bodied children to those with limited mobility, the literature identifies increased parental stress in the latter group, however confounding factors such as family functioning and the child's adaptive skills must be considered in complex conditions such as spinabifida (Ong et al., 2011). The fact that the increased care needs and time required by a child with a physical disability causes long term stress to parents, has been identified as having a negative impact on the child themselves, who may experience increased stress and display disruptive behaviour (Song et al., 2015). The authors also suggest there is a need for improved paternal education for fathers of physically disabled children to enable more shared carer responsibility, thus potentially reducing maternal stress. The study suggests that mothers would welcome greater input from their child's father, which they feel would benefit the whole family in addition to reducing their own stress. The current study explored only maternal emotions of children with LSL, thus presenting a limitation to the study, but identifying an opportunity for future research.

Knowledge and understanding about the needs of parents in supporting their child and how they can encourage their child to participate in meaningful activities both within and outside the school environment needs to be encouraged, to enhance both child and parent well-being.

7.6.3. Pain

There were 28 children (51%) in the current study who reported pain, but very high levels of pain were uncommon (as reported in chapter 4). The literature suggests that parents often under report and fail to recognise their child's pain unless it is severe, particularly in young children and that this results in untreated pain (Clancy et al., 2005). This may partly explain the results from the current study, whereby there was a trend, but no statistically significant finding, towards higher anxiety and depression scores in parents of children in pain.

There was however an association between binary pain scores and the Medical Care domain of the PIP scale. The current study highlighted medical procedures as including Clean Intermittent Catheterisation (CIC), with 25

children (44.4%) requiring CIC. CIC is recognised as causing discomfort and research continues to produce a urinary catheter that causes less discomfort than the ones currently in use (Johansson et al., 2013). Holroyd described urinary catheterisation as an invasive procedure with associated pain and trauma for the patient, and presents an increased risk of urinary tract infection (Holroyd, 2016).

The requirement for parents to undertake painful medical procedures on their child is traumatic to both child and parent. Rankin et al investigated the parental experience of managing their child's frequent insulin injections and identified that both child and parent were distressed and upset when the insulin injection had to be administered due to the pain inflicted by the parent (Rankin et al., 2015). The authors suggest that administrating their child's injection can potentially have an adverse effect on the parent's quality of life and that further research is required to more fully capture the burden to parents, of inflicting a painful procedure on their child.

Increased pain in children often correlates with increased parental stress and the current study findings indicated that although not statistically significant, PIP ratings were consistently higher in parents of children who experienced pain. Similar findings were identified in the literature, including a review by Cousino and Hazen of parenting children with a variety of chronic illnesses (Cousino and Hazen, 2013), and in studies of children with sickle cell disease (Barakat et al., 2007) and rheumatoid arthritis (Anthonya et al., 2011); however all authors highlighted the importance of considering confounding factors in their studies, including co morbidities associated with the child's disease, family income and socio economic status.

The literature suggest chronic pain in children can have multiple effects on parents: Claar et al (Claar et al., 2008) suggest that when facing their child in chronic pain, parental responses can be maladaptive, for example discounting the child's pain, or responding in an inappropriate way, and that this in turn may result in increased stress and disability for the child and as a consequence, increased stress for the parent; catastrophising thoughts and beliefs about their

child's pain can result in restriction of the child's activity and engagement (Caes et al., 2011), whilst parental overprotection can cause the child additional impairment, somatic symptoms and can influence school functioning (Claar et al., 2008, Logan et al., 2012). Furthermore, family beliefs, knowledge, expectations, normal coping responses and the degree of disempowerment perceived, can all contribute towards how a parent supports and manages their child in pain (Gaughan et al., 2012). In addition to increased levels of parental stress associated with chronic pain in their child, Palermo identified changes in parenting behaviour and that marital adjustment occurred in many families (Palermo et al., 2008, Palermo et al., 2014). Poorer family functioning in children with pain related disability has been suggested by Lewandowski et al (Lewandowski et al., 2010), with greater conflict, less cohesion and increased stress within the family.

Karlsson et al (Karlsson et al., 2014) suggest that a fundamental role of parenting is to protect the child and therefore, when facing their child in pain, parental ability to provide support can be reduced. The results from the current study provide a preliminary exploration of the emotional effect that chronic pain in children with LSL has on their parents, and identified that greater understanding and support is required for this group of parents.

7.6.4. Urology

The literature suggests there is a relationship between urinary dysfunction and parental emotion yet this has not been previously evaluated in children with LSL. In the current study there were 37 children (68%) with abnormal urological function, of whom 13 (24%) were deteriorating. The results identified a statistically significant correlation between the parental HADS Depression domain and child urology ($p=0.04$, Spearman's correlation) and statistically significant correlations between all domains of the PIP scale with the exception of Role Function.

The latter point is not supported in the literature; a systematic review of parenting stress among parents of children with a variety of chronic illnesses,

identified that extended parental roles and responsibilities were associated with disruption to the parents' ability to care for other members of the family and their own ability to work (Cousino and Hazen, 2013). The authors suggested that increased stress was caused not by the duration and severity of the child's illness, but by extended parental responsibility for undertaking medical procedures. In the current study, there were 25 children (44.4%) using Clean Intermittent Catheterisation (CIC.) Undertaking CIC on their child constitutes an extended parental role which requires a strict adherence to a timetable; this can be disruptive and stressful not only to the child, but to the parent who may have to attend school regularly to undertake the procedure. CIC has been shown to cause more parental stress than urinary incontinence, often due to the young child's dislike of the procedure, pain inflicted on insertion of the catheter in some children and non-compliance in adolescents, the latter point adding to parental stress regarding potential upper urinary tract damage (Kanaheswari et al., 2011, Nejat and El Khashab, 2011). The authors suggest that undertaking CIC in a non-complaint child is an abuse, disrupts the normal child- parent relationship and is extremely distressing for both child and parent. There was no association in the current study between Role Function as measured by the PIP scale and urology, but in light of the findings in the literature, a tool more sensitive to the effect on parents of undertaking CIC may provide more specific data. The current study identified an association between the HADS depression domain and urology and similar findings were found in the literature: In a study of 81 parents of children with spinabifida, parental depression was associated with managing their child's urology and related specifically to undertaking CIC (Kanaheswari et al., 2011). The authors highlight the importance of considering the child's reaction to CIC, in addition to the status of the caregiver including single parenthood in the overall assessment of parental depression.

There were 14 children (25%) in the current study with urinary incontinence, but mitigating factors may contribute to incontinence in young children. The youngest children in the current study were aged 5 years and although urinary continence is usually achieved by this age, hospitalisation, invasive investigations and the need to undertake CIC may all have contributed to disrupted behaviour and as a consequence, delayed achievement of urinary

continence. Additional factors influencing a child's ability to achieve continence include the individual child's developmental readiness and parental expectations (Wu, 2010).

The stigma attached to urinary incontinence is highlighted in a study by Fischer et al, who explored the impact of incontinence on the lives of children and their parents (Malm-Buatsi et al., 2015). The authors identified that the effect of urinary incontinence varies from a minimal disturbance, to isolation and social rejection of the child and correspondingly increased stress to the parent. The authors also suggested that children and parents may differ in their attitudes towards incontinence, with some parents encouraging the child to achieve continence before the child is developmentally ready to do so, thus increasing conflict and stress to both themselves and the child.

The current study was not specifically designed to evaluate factors related to CIC and the questionnaires used did not encompass this domain; however, a study of children who undertake CIC with different pathologies from the study group, demonstrated that parental depression was associated with the need for the child to undertake CIC (Kanaheswari et al., 2011); similar studies may enable more understanding and meaningful findings of the effect that undertaking CIC in children with LSL, has on their parents.

7.6.5. Bowel function

The literature suggests there is a relationship between bowel dysfunction and negative parental emotion (Kaugars et al., 2010), but this has not been previously published in relation to children with LSL. There were 24 children (44.4%) in the current study group with abnormal bowel function of whom 5 (9.3%) had deteriorating function.

Fagerskiold and Glad Mattsson (Fagerskiold and Glad Mattsson, 2010) suggest that children with bowel and / or bladder dysfunction rely more on support from their parents and carers when compared to children of the same age who have normal bowel / bladder function, and that this reliance increases the carer

burden and associated stress. However, there was no association identified between the child's abnormal bowel function and parental anxiety and depression on the HADS score, or any of the domains on the PIP scale in the current study. This finding is surprising, since the literature suggests that multiple domains of family functioning are disrupted by a child's faecal incontinence, including necessitating changes to the family routine, reduced parental leisure time and increased costs associated with laundry (Kaugars et al., 2010). Additional factors to consider when assessing the impact of abnormal bowel function include sociodemographic and socio economic status (Guilfoyle et al., 2012), in addition to the medical and practical requirements (Cushing et al., 2016); these factors have not been assessed in the current study and suggest a limitation in the results.

In addition, the current study findings suggest that a more precise tool to measure the effect on parents of managing bowel dysfunction in children with LSL is required, to ensure there are no gaps in the provision of support to parents.

7.6.6. Non clinical factors

A scoping review examining parental supportive needs when caring for a child with a rare illness identified multiple needs, including additional information regarding the illness, addressing the associated uncertainties, addressing social and emotional needs, and the need for increased support for parental caring responsibilities (Pelentsov et al., 2015).

Several additional factors have been identified in the literature relating to the emotions of parents caring for a child with a chronic illness. Whilst these have not been captured by the HADS and PIP questionnaires in the current study and as such, present a limitation, they are discussed in the following section as they contribute towards parental well-being.

7.6.6.1. Financial burden

The financial burdens of caring for a child with a chronic disease are many and include attending hospital appointments, costs related in taking time off work, care for siblings and transport costs. Palermo et al (Palermo et al., 2014) suggest financial burdens increase parental stress and should be considered in the overall management of the child and family. Financial implications and the burden associated with caring for a child with LSL have not been explored and suggest the need for further studies to evaluate if such burdens exist, and what support may be beneficial.

7.6.6.2. Disease uncertainty

Disease uncertainty can be a cause for parental stress and anxiety. The disease trajectory of LSL in children is unknown and parental uncertainty related to their child's illness was discussed in chapter 6. The current study showed that 48 parents (88.8%) identified uncertainty about the future as an important concern; these concerns were similar across LSL groups. The main areas of uncertainty included the risk of clinical deterioration, the possibility of future surgery and longer-term issues including perceived barriers to work. Disease trajectory in rare diseases is difficult to project and Gray et al (Gray et al., 2013) identified the need for assessing parental stress associated with caring for a child with a rare, chronic illness, particularly where disease progression was intermittent and unpredictable.

7.6.6.3. Parental hope and well-being

Feizi et al (Feizi et al., 2014) explored parental responses to managing children with disabilities and identified that anxiety about the future and the loss of their hope for their children, contributed to a reduction in their own mental health. In a study exploring the parenting of children with spinabifida, hope was identified as an important factor in parental well-being and was also associated with improved HRQL in the child (Kirpalani et al., 2000); the authors suggest a prospective study may prove useful in identifying a causal relationship between

parental hope and parental well-being. Parental hope is little explored in the literature but would provide an interesting project for future research in parents of children with LSL.

7.7. Limitations and suggestions for future research

7.7.1. Parental gender

Gender may be a factor when assessing parental response to a child's chronic disease, with mothers showing a higher level of anxiety and depression than fathers (Vermaes et al., 2007). In the current study, mothers completed all the questionnaires, thus the association between parental gender and PIP and HAD questionnaires could not be analysed. Future studies should include paternal assessments of PIP and HAD to ensure paternal needs are addressed.

7.7.2. Family centred care

Family centred care is important in the management of a child with a chronic illness with perceptions regarding the child's illness and the impact this has on family organisation sometimes impacting on the disease trajectory (Cipolletta et al., 2015). The impact on, and the influence of family centred care have not been explored in this thesis and future studies are recommended to provide further understanding of this theme. The PedsQL™ family impact module (FIM)(Varni et al., 2004) measures the impact that a child's chronic illness has on the parents and family, including the effect on family activities and family relationships. When results from the FIM were correlated with results from the HADS scores, Medrano et al (Medrano et al., 2013) identified that higher levels of parental anxiety and depression were associated with lower parental HRQL and family functioning. Using the FIM to assess the parents and families of children with LSL in future studies would advance our understanding of the needs of this population.

7.7.3. The child's age

The child's age may be a factor when assessing parental response to a child's chronic disease. The parents of younger children with spinabifida described a higher anxiety and protectiveness of their children, than parents of adolescents with spinabifida (Malm-Buatsi et al., 2015). The authors suggest that parents with children less than 4 years old had higher levels of anxiety and stress. This finding was reiterated in a study by Streisand et al, who explored the anxiety and depression of parenting a child with diabetes; whilst acknowledging the potential multifactorial causative factors of stress, the authors suggested that parents of young children were at an increased risk of stress than those of older children (Streisand et al., 2001). Confounding factors would need to be considered in future studies and should include the following: the disease trajectory, the child's ability / lack of ability to achieve urinary and bowel continence particularly in the school environment, a new requirement to undertake CIC, a realisation that normal physical milestones may not have been reached, and that the child may not be able to undertake the activities that school provides. Future studies analysing the emotions of parents of children with LSL might include the ages of the children, with the aim of addressing parent and child health outcomes; however larger numbers of participants and exploration of confounding factors would be beneficial in providing meaningful data.

7.7.4. Ethnicity and socio economic status

Holmberg and Devine suggest ethnicity and socio economic status play an important role in parental response to their child's illness (Holmbeck and Devine, 2010) and that parents of children with spinabifida from lower socio-economic status are at increased risk for psychosocial difficulties. Ethnicity and socio economic status have not been assessed in the current study and longitudinal studies should be considered to address and improve psychosocial outcomes for parents of children with LSL.

7.7.5. Parenting characteristics

How parents make everyday decisions about their child's behaviour and discipline, parenting skills and marital status, are all factors that influence parental well-being and family functioning and suggest further research to include these factors may help our understanding of parents of children with LSL.

7.7.6. Disease trajectory

The trajectory of a child's chronic illness can require parental adjustment in terms of care requirements and may cause fluctuations in parental emotional response including stress (Vermaes et al., 2007). The current study measured parental response at one point in time and longitudinal studies may provide more information and highlight resource and intervention requirements for this group of parents.

7.8. Summary

Although there was a trend for parents of children with LSL to have increased anxiety and to a lesser degree depression when compared to parents of healthy children, urology and pain were the only domains that were associated with increased anxiety and depression. Motor, urology and pain correlated significantly with almost all domains of PIP ratings. Parents of children with transitional lipomas were found to be at higher risk of stress and anxiety and support could be risk stratified to this group of parents.

The current research sought to provide an initial understanding of parenting stress and mood of parents of children with LSL. Demanding medical regimes, adherence to these regimes, pain and school reintegration have been associated in the literature with increased parental stress and anxiety (Cousino and Hazen, 2013). Longitudinal research is needed to further understand how and why emotions and responses may vary over time in children with LSL. The diagnosis of a rare condition can be a burden for the parent and family, and

parental well-being is an important part of the child's care as they are usually the primary care giver.

The impact on the parents of children with LSL has not been previously explored and this thesis has provided a preliminary description of this impact and identified gaps in our current knowledge. Improved understanding of the emotions of parents of children with a rare, chronic illness such as LSL may assist in directing future interventions and supportive care, including financial and psychosocial resources aimed at improving parental functioning and well-being, and as a consequence, improved child health outcomes.

To conclude, renewed research focusing on parents of children with LSL is warranted. By identifying and understanding those factors which increase parenting stress, depression and anxiety, effective interventions and support can be developed to improve parent functioning and well-being.

The following chapter presents a conclusion to the thesis.

Chapter 8. Conclusions and implications

8.1. Introduction

The final chapter draws upon the entire thesis to address the main aims of the thesis and identify areas for further research.

The two general aims of this thesis were to:

1. Develop a tool for assessment of children with lumbosacral lipoma (LSL) in the outpatient setting. It is hypothesised that this assessment tool will:
    a. Provide a standardised method of continual surveillance of the child including Health Related Quality of Life (HRQL).
    b. Provide a standardised method by which to objectively assess outcomes of intervention including surgery.
2. Describe a preliminary analysis for determining if there is a relationship between LSL type and clinical and HRQL outcomes. In addition to providing a more thorough understanding of this rare condition, this information may assist in the evaluation of prognosis and inform a more selective treatment policy. The data were then used to identify what particular aspects of quality of life appeared to be important to the child with LSL.

In addition, this thesis describes a preliminary analysis of the impact of LSL on the parents of children with LSL.

Steps taken to achieve these aims included:

1. Undertaking a systematic review (SR) to identify the salient clinical symptoms and signs on which to base decision making regarding interventions including surgery.
2. Objectively assessing a group of children with LSL using standardised methods of clinical assessment.

3. Evaluating the child's Health Related Quality of Life (HRQL). This was divided into the use of formalised questionnaires and by asking the children what was important to them in terms of their disease.
4. Distilling the study findings from steps 2) and 3) into an assessment tool which can be used within the outpatient setting to provide a consistent and overall assessment to provide a basis for future management.
5. Distilling the study findings from the HRQL questionnaires and the responses from the patients to give a more holistic view of the burden of the disease. This would also be used to provide a basis for a management plan.
6. Identifying if there is a relationship between LSL type, and clinical and HRQL outcomes.

Validated questionnaires were used to explore the impact of LSL on the parents of children with LSL; in addition, the parents were asked what was important to them in terms of their child's disease.

In addressing the study aims, the preceding chapters have provided a comprehensive overview of LSL including symptomology and assessment methods, and identified associations between LSL types and outcomes; finally, the effect this disease may have on parents has been explored.

This final chapter summarises the main findings of the thesis and discusses the implications of the findings including potential policy implications. The limitations of the research are discussed and suggestions for further research made.

8.2. Main findings

The thesis started with a description of the pathoembryology of LSL and the pathophysiology of clinical deterioration in children with LSL and its clinical correlate; as a consequence, the salient clinical signs and symptoms on which to base management and decision making can be more thoroughly understood. NHS England have specified the importance of safe and standardised practice in paediatric neurosurgery (NHS England, 2015), and as a consequence a

systematic review was undertaken to identify what standardised assessment methods are currently used to assess children with LSL, both nationally and internationally. As chronic disease in childhood is often associated with a reduced HRQL and has the potential to lead to poor mental health in adulthood (Austin et al., 2016), HRQL was also included in the review process.

8.2.1. The systematic review

The literature reflected the challenges in assessing and scoring clinical symptoms in children with LSL; in addition, the findings from the review suggested that methods of clinical evaluation were not standardised, were non validated and difficult to apply. The majority of papers discussed the importance of early recognition of clinical deterioration in children with LSL. Despite this, and the fact that most children once deteriorated will seldom return to their baseline status (with the exception of pain and to a lesser extent, neurology), there was a lack of standardised assessment tools identified by which to monitor the individual child, and minimal reference to the HRQL of children with LSL.

In addition, there was paucity of literature identified, relating LSL type and clinical and HRQL outcomes; such information might enable the clinician to offer a potential prognosis for the individual child and family, and risk stratify treatment plans.

As a child cannot be viewed as an individual unit but as part of a family unit, the effect that LSL has on the child's parents was also explored.

Following the systematic review, the thesis aims were identified as above.

8.2.2. The reasons for standardising practice

One of the aims of NHS England is to improve the health outcomes for children undergoing neurosurgery across the UK through the implementation of nationally accepted standards, standardised care and transparency of published

outcomes (NHS England, 2013/14, NHS England, 2015). Contrary to the aims of NHS England, the systematic review in chapter 2 identified that there was a variation in, and a lack of consensus, about what should be assessed and how this should be undertaken. Outcomes reported in the current literature and highlighted in the review, focused predominantly on surgical outcomes and the continuing controversy between the management of symptomatic and asymptomatic children.

As discussed in Chapter 1, the Society of British Neurological Surgeons (SBNS) and the British Paediatric Neurosurgical Group (BPNG) aim to reflect the distribution of national neurosurgical activity as a step towards the provision of more specific audit and outcomes, both within adult and paediatric neurosurgery. It is envisaged that these steps will encourage standardisation of practice and a reduction in variation, and that this will assist in audit data collection and in improved outcomes for patients.

Variations in surgical expertise and techniques are however difficult to measure, analyse or reproduce across institutions, with potential bias in the publications of results. The rarity of children with LSL presents difficulty in analysing data from publications from single clinicians and single institutions, where the data are retrospective and uncontrolled (Wykes et al., 2012). Regardless of variations in surgical techniques, there remains a requirement for standardisation of assessment as highlighted by the SBNS and PBNG.

The importance of evaluating and managing the HRQL of individuals with chronic disease and the effect that has on their carers, was highlighted in The NHS Outcomes Framework (Department of Health, 2014 / 2015). The current study results identified the importance of evaluating the HRQL of children with LSL, as reduced HRQL may: i) have immediate effects on the child's well-being; ii) have potential longer term effects that impair transitioning through to adulthood and iii) increase the financial burden to the NHS, if not addressed.

Based on the lack of standardised assessment of children with LSL identified in the systematic review including the assessment of HRQL, and the requisites

identified by NHS England for standardisation of practice, the thesis aims were identified and the following methodology undertaken.

8.2.3. Research methodology

To understand which clinical variables and HRQL measures should be included in an assessment tool, and the method by which these might be collected, 54 children were assessed in the outpatient setting (chapter 3 provides detailed methodology including statistical analysis) and results from clinical data (including LSL type and the presence of a syrinx) and HRQL measures, were collected.

In addition, to understand the effect that LSL may have on parents, all parents were asked to complete two questionnaires and were asked what was important to them in terms of their child's disease.

The associations between LSL type, and clinical and HRQL outcomes were explored, with the aim of identifying those children most at risk of deterioration.

8.2.4. Results

8.2.4.1. LSL type and clinical outcomes

The association between LSL type and individual clinical variables without confounding factors has not been previously examined. Novel findings in the thesis identified that clinical parameters were not equally distributed across LSL types and that there was an association between LSL type and specific clinical outcomes. The transitional group with associated rotation of the spinal cord and shortening of spinal nerve roots, were found to be at the highest risk of clinical abnormalities; these included an increased prevalence of urological dysfunction and increased requirement for Clean Intermittent Catheterisation (CIC), a higher rate of abnormal neurology and abnormal orthopaedic status, and an associated reduction in physical activity and increased pain, when compared to the caudal and dorsal group. In addition, the complex abnormal anatomy of this

group of lipomas was further increased by the higher incidence of a syrinx. The presence of a syrinx was associated with increased abnormal neurology but with no other clinical variables.

In line with the literature (Pang et al., 2010, Wykes et al., 2012), the current research identified more females than males in the study group; in addition, females were in the majority for each of the LSL types in the current study and this was of statistical significance for the dorsal group. Gender did not however, exert a significant effect on categorical clinical outcomes.

The study results suggest children could be risk stratified according to lipoma type, with more intensive initial investigations and potential selection for surgery in children with transitional lipomas and the presence of a syrinx.

8.2.4.2. LSL and HRQL

The current study is the first to examine the HRQL of children with LSL and identified that children with LSL have a lower HRQL than healthy children in several domains, and specifically in relation to the presence of pain, urological abnormalities and physical limitations. Chapter 6 explored what was important to the child in terms of their disease. This novel approach, despite the difficulties in extrapolating specific data in a complex medical condition, identified the importance of urological function in regard to the child's HRQL, and highlighted the importance of future research including the need to assess what specific elements of urinary dysfunction relate to a reduced HRQL. The systematic review published by Sawin and Bellin (Sawin and Bellin, 2010) identified the lack of instruments by which to measure abnormal sphincter function in children with spinabifida and the authors suggest the need for a questionnaire specific to continence. The spinabifida health-related quality of life questionnaire (HRQOL) (Parkin et al., 1997) was adapted to incorporate a Supplementary Continence Questionnaire (MacNeily et al., 2009). The results from the latter study however showed little association between HRQL and achieving continence, although the authors suggested limitations to their study included the use of subjective interviews and a non-validated scoring instrument.

A further study comparing the HRQL of children who undertook Clean Intermittent Catheterisation (CIC) and the HRQL of healthy children, identified that the HRQL was similar across the two groups (ALPERT, 2005) No recent studies were identified comparing the HRQL of children who undertook CIC with those who had no urological abnormalities; neither were any found which compared the HRQL of children undertaking CIC with those with urinary incontinence who were not undertaking CIC. The impact of managing urinary dysfunction requires further investigation before an association between specific elements of urinary dysfunction and HRQL can be confirmed.

Results from this thesis also identified that pain and physical limitation were associated with a reduced HRQL in children with LSL. Improved pain management should become a priority in managing children with LSL: Chronic pain is recognised as having a negative impact on the child's HRQL with low ratings in both physical and psychosocial HRQL scores (Young et al., 2013) and in addition, may result in parental absenteeism from work and added financial burden. Environmental barriers (including school) which may present a challenge to children due to their physical limitations and contribute to reduced participation in activities, need to be addressed further to enable the implementation of appropriate support, including policy changes.

LSL is a lifelong condition and evaluation of HRQL can be used as an outcome measure to complement objective clinical measures, by assessing and understanding the impact of a chronic disease (Bekesi et al., 2011).

Juth et al (Juth et al., 2008) suggest that self-esteem predicts HRQL, with low self-esteem resulting in a lower HRQL; there was an association between self-esteem and some of the examined variables in the current study, with a trend for the transitional group to report lower self-esteem. However, there were few findings of statistical significance and the measurement of self-esteem (as measured by the PH2) was not included in the tool development.

8.2.4.2.1. LSL type and HRQL

The results identified that children with transitional lipomas had a lower HRQL than those with caudal and dorsal lipomas and for most domains, those with dorsal lipomas had the highest HRQL.

By identifying the lipoma group most at risk of a reduced HRQL together with the clinical abnormalities most associated with a reduction in HRQL (urological abnormalities, physical limitations and pain), resources can be targeted more effectively.

8.2.4.3. LSL type, clinical and HRQL outcomes

Children with transitional lipomas are at an increased risk of clinical deficits and reduced HRQL. This suggests that risk stratification is important for children with LSL to provide the clinician with information on which to base his / her management plan, provide a potential prognosis for the clinician, children and their parents, and to guide commissioners with regard to resource and policy requirements.

8.3. The development of an assessment tool

8.3.1. The rationale for an assessment tool

The requirement for standardised practice and assessment has been discussed, and the lack of such assessments identified in the systematic review. The use of the Necker-EnfantsMalades scale (NEM) (Kulkarni et al., 2004a, Pierre-Kahn et al., 1997) and The Clinician's Assessment form (May et al., 2013) were both used to document data as described in chapter 4 but as discussed, both were found to be lacking in detail, with neither chart providing sufficient information on which to base a management plan.

The mission statement following the NHS England Safe and Sustainable Paediatric Neurosurgical Review was as follows: "Safe, sustainable and world class. Not ordinary, OK or just good enough" (NHS England, 2013/14). The aim

following the review has been not only to produce and adhere to agreed clinical standards, but also to reduce variations in practice and outcomes (Young, 2014). NHS commissioners have identified the need for a uniform, multidisciplinary approach to children with spinal dysraphism including LSL. The development of The GOS Clinician's Assessment Tool (suggested acronym G-CAT) is a step towards achieving this.

8.3.2. What should be included in the G-CAT

The systematic review and the results from the thesis identified that neurology, urology, orthopaedic and bowel status should all be assessed, and that standardised physiotherapy assessments including tone, muscle strength and reflexes in line with MRC guidelines (MRC, 1981), and standardised urodynamic assessments should be utilised; in addition, the presence, severity and location of pain should be assessed. All variables should be rated in terms of normal (no deficit), abnormal but stable status, and deteriorating status, thus providing the clinician with longitudinal data on which to base his/ her management plan for the individual child, and to compare results in multicentred research. HRQL should be assessed in conjunction with clinical assessment to form a holistic management plan.

8.3.3. The development of the G-CAT

Clinical assessment remains the optimum method of evaluating and monitoring symptoms in terms of stability, improvement or deterioration, in relation to the developmental milestones of childhood. The requirements of an assessment tool must include ease of use, reproducibility and comparability. Findings from the thesis have provided a strong foundation on which to base the proposed G-CAT, with data from the systematic review and the clinical assessment of 54 children with LSL used in the preliminary development of the tool. The aim is to provide consistency when assessing children with LSL, regardless of the disease trajectory.

The importance of pain in heralding potential tethered cord was identified in the study and the Paediatric Pain Questionnaire (PPQ) (Varni et al., 1987) found to be an appropriate tool by which to assess pain in children with LSL: the questionnaire provides a numerical score by which to monitor pain and evaluate the effectiveness of pain interventions, and has the added benefit of providing a body outline drawing which the study identified as an effective method for the child to describe the severity and position of his / her pain; the use of colour to depict pain provides a further preliminary insight into how children perceive and describe pain. The PPQ was therefore included in the G-CAT.

The PedsQL (Varni et al., 2001) was found to be a validated, appropriate method of assessing the HRQL of children in the study group and consequently was included in the G-CAT.

8.3.3.1. The importance of the red flag system.

The results of the study provide a step towards developing a red flag system. A red flag system provides an alert by which clinicians including Allied Health Professionals (for example community physiotherapists) can identify subtle deterioration in clinical symptoms using the new assessment tool, and expedite a rapid referral to the neurosurgeon if required. Red flag systems were first introduced when managing back pain in the community setting, to help non specialists differentiate between serious and non-serious illness (Welch, 2011). The red flag system has since been expanded to cover many aspects of health care practice to assist in identifying the potential presence of a serious condition, which could lead to irreversible morbidity if untreated / treated inappropriately. The development of the G-CAT incorporates the proposal of a numerical scoring system (a red flag system) on which to base management plans.

8.3.3.2. The GOSH Clinician's Assessment Tool – G-CAT

The G-CAT for use with children with LSL will provide guidance for the clinician through the use of a numerical scoring system. A score will be attributed to the clinical status of the child as previously discussed, with a score of 1

representing normal function, a score of 2 depicting abnormal stable status, and a score of 3 depicting deteriorating function, on each of the 4 domains (motor, pain, urology, bowels). Thus a maximum total score indicating deterioration in function in all 4 domains =12, and a minimum total score indicating the absence of any abnormality in any of the 4 domains, = 4.

A child will be scored as clinically deteriorating according to the following criteria:

1. Motor: If there is evidence of gait or motor deterioration (i.e.: Muscle strength, reflexes and level of physical activity).
2. Pain: If there is evidence of increased pain in the legs / back and legs, particularly related to physical activity.
3. Urology: If there is evidence of deterioration as confirmed by standardised urodynamic studies and assessment.
4. Bowels: If there is evidence of increased constipation / soiling / incontinence.

The following criteria for suggested management based on a numerical system is in the preliminary stages, and will require validation.

- If the total score of all domains is between 5 and 10, then surveillance only is required (if there are no individual scores of 3; any such scores would necessitate further investigation).
- If there are any individual scores of 3, then surgery for resection of lipoma / spinal cord untethering should be considered.

The G-CAT is presented in figure 8.1.

Great Ormond Street NHS Hospital for Children
NHS Foundation Trust

Lipoma type
☐Chaotic ☐Transitional ☐Dorsal ☐Caudal
Syrinx ☐Yes ☐No
Surgery ☐Yes ☐No

Date
Insert sticker here

		Date	
		Date	
		Date	
G-CAT			
Motor function			
	Muscle charting (MRC)		
		Hip flexors L3	Right ☐ Left ☐
		Knee extensors L4	Right ☐ Left ☐
		Ankle extensors L5	Right ☐ Left ☐
		Long toe extensor S1	Right ☐ Left ☐
		Ankle plantar extensors S2	Right ☐ Left ☐
Summary muscle score			☐ *
	Reflexes		
		Patella L3	Right ☐ Left ☐
		Achilles S1	Right ☐ Left ☐
Summary reflex score			☐ *
Summary motor score			☐ *
Pain			
		Pain on activity	Yes ☐ No ☐
		Pain in leg(s)	Yes ☐ No ☐
		Pain in back and legs	Yes ☐ No ☐
Summary pain score			☐ *
Urology function			
		Drbbling	Yes ☐ No ☐
		Urgency	Yes ☐ No ☐
		CIC	Yes ☐ No ☐
		Mitrofanoff	Yes ☐ No ☐
	Hyperlink to BFA		Yes ☐ No ☐
Summary urology score			☐ *
Bowel function			
		Constipation	Yes ☐ No ☐
		Soiling / incontinence	Yes ☐ No ☐
		ACE	Yes ☐ No ☐
Summary bowel score			☐ *
Orthopaedic function			
		Normal mobility	Yes ☐ No ☐
		Early fatigue	Yes ☐ No ☐
		Orthotic use	Yes ☐ No ☐
		Wheelchair user	Yes ☐ No ☐
Summary orthopaedic score			☐ *

Scoring guide *

1. Normal for age
2. Abnormal stable
3.Deterioration

Patients were considered to have deteriorated if:

- Motor: If child has developed evidence of motor or gait deterioration
- Pain: Increased pain in leg / back (particulary on activity)
- Urology: Deterioration as confirmed by urological assessment
- Bowel function:Deterioration in constipation / soiling

Figure 8.1 Assessment tool (G-CAT) for children with LSL

HRQL	PedsQL PPQ	Score (0-10)
PedsQL PPQ pain rating	*Child Pain Now*	
	Child worst pain previous week	
	Parent rating of child's pain now	
	Parent rating of child's worst pain previous week	
PedsQL Child		Score (0-100)
	Physical	
	Emotional	
	Social	
	School	
	Total	
PedsQL Parent		Score (0-100)
	Physical	
	Emotional	
	Social	
	School	
	Total	

Figure 8.2 HRQL and PedsQL PPQ for use with G-CAT

8.3.4. The next steps

8.3.4.1. Tool validity, reliability and the audit process

For the G-CAT to be used as a research tool, it must first be tested for validity and reliability. This complex process includes, amongst other processes, ensuring the tool is fit for purpose and that it measures what is required, minimising errors in the measurement process (validity), that is responsive (able to detect change over time in the area of interest), has been tested for inter reliability (observer agreement) and that the methods of assessment are stable (thus measuring reliability). The question whether all components should be equally weighted in calculating the final score needs to considered.

The G-CAT will first be utilised and audited in the study centre, with 2 paediatric neurosurgeons with a special interest in spinal surgery, completing the form independently over a three-month period. The study will be registered in line with the Trust audit policy of the study site, which has the added benefit of providing advice on undertaking such projects. The audit process will test for observer agreement, validity and responsiveness. The additional aim of the

audit will be to identify barriers and facilitators in completing the G-CAT including the form design, usability and time to complete. Testing of the G-CAT requires assessment of the feasibility of its use in the busy clinic setting, not just to clinicians but also how acceptable the HRQL and PPQ pain measures are to families and children. The HRQL (PedsQL) and PPQ pain measurement questionnaires are validated questionnaires but an advisory group is proposed to include parents and children to examine issues including optimal timing for distributing and collecting HRQL information, and ensuring there are no gaps in the data collected. The presence of children and families in the advisory group has the added benefit of providing information about their experience of living with the disease, and can assist in the potential future development of a tool which captures the parent experience.

Following the G-CAT audit at the research study centre, the form will be altered accordingly and then distributed to paediatric neurosurgeons in the UK with a special interest in spinal dysraphism. They will be asked to complete the G-CAT for a period of a year, noting which qualities are most discriminatory, (if these have not previously been addressed), for example differentiating between minimal pain and severe pain, and between urinary continence and incontinence. One of the audit aims is to identify if the tool is responsive to clinical change and this highlights the importance of using standardised assessment measures in identifying changes in clinical status that are clinically meaningful.

The neurosurgeons will be asked for any comments relevant to the G-CAT and whether they feel it captures the necessary assessment criteria. Changes to the tool can be made accordingly and the revised tool can be repiloted. By engaging stakeholders, these professionals will be well informed about the tool and can promote use of the G-CAT among the wider neurosurgical community for example through the BPNG and SBNS. Once validated, the G-CAT could become standardised practice in managing children with LSL.

In addition to the development of the G-CAT and incorporating the red flag into the tool development, an algorithm for management of children with LSL has been developed and is provided in figure 8.2.

8.3.5. Proposed algorithm / pathway for management of LSL

The thesis results, in addition to identifying the requirement for a standardised assessment tool, highlighted the importance of differentiating between LSL types and their clinical correlates. The importance between those children who are stable with a fixed deformity and those who are deteriorating was identified in the study, and an algorithm suggested for management of all children with LSL.

The requirement for care pathways for children undergoing neurosurgery was proposed by The NHS Specialised Services standards document “Children’s Neuroscience Network (for the Neurosurgical Child) Specification Standards” (NHS England, 2013/14). With regard to spinal dysraphism, the commissioners require the publication of surgical outcome data including mobility status and sphincter continence at 1 and 5 years post-surgical intervention. The commissioners suggest a multidisciplinary approach to the child’s care, specific guidelines for urodynamic assessment and standardised physiotherapy assessments measuring muscle strength before and following surgical intervention. Fundamental to care pathways is the need to have a tangible measure of outcome.

8.3.6. Suggested NHS outcomes for children with spinal dysraphism, resulting from the study

Specific to spinal dysraphism, NHS commissioners proposed the publication of surgical outcome results with regard to mobility status and sphincter continence at 1 and 5 years post-surgical intervention (NHS England, 2013/14). They suggested specific guidelines to assess and monitor the upper and lower renal tracts of this group of children and standardised physiotherapy assessments measuring muscle strength before and following surgical intervention.

The thesis provided a snapshot view of a cohort of children with LSL, and demonstrated that such a snapshot provided useful information regarding the outcome status of these children, regardless of their management / surgical trajectory. As a consequence, the following NHS outcomes are suggested for children with spinal dysraphism, including LSL. These outcomes are deliberately simple and broad, with the aim of encompassing the wide spectrum of associated clinical deficits.

It is proposed that children should have an assessment at 10 years and 16 years of age. Assessment at 16 years of age will address the NHS England key service outcome regarding transition into adult services. Poorly planned transition can lead to absence of follow up in addition to increased non adherence to treatment protocols (for example CIC), with potentially increased serious morbidity.

NHS England outcomes also state that the HRQL of individuals with chronic conditions must be assessed and addressed, in addition to the well-being of parents / carers. Ensuring continuity of care across health and social care is essential for children with spinal dysraphism and their carers, and promotes well-being and quality of life.

The thesis identified mobility, pain, urology, bowel function and HRQL as important in assessing outcomes for children with LSL and the author suggests the same domains may be applied to children with spinal dysraphism, plus the addition of a transition key outcome for children aged 16 years.

The following tables provide suggested outcomes for children with spinal dysraphism.

Table 8.1 The percentage of children at 10 years of age with the following:

Domain	Number of children reviewed	Percentage of children
Reduced mobility		
Urological deficits		
Undertaking CIC		
The presence of pain		
Deficits in bowel function		
Reduced HRQL		

Table 8.2 The percentage of children at 16 years of age with the following:

Domain	Number of children reviewed	Percentage of children
Reduced mobility		
Urological deficits		
Undertaking CIC		
The presence of pain		
Deficits in bowel function		
Reduced HRQL		
Transition plan in progress		

Following on from the current study, a preliminary algorithm has been developed for the management of children with LSL and is provided below in figure 8.3.

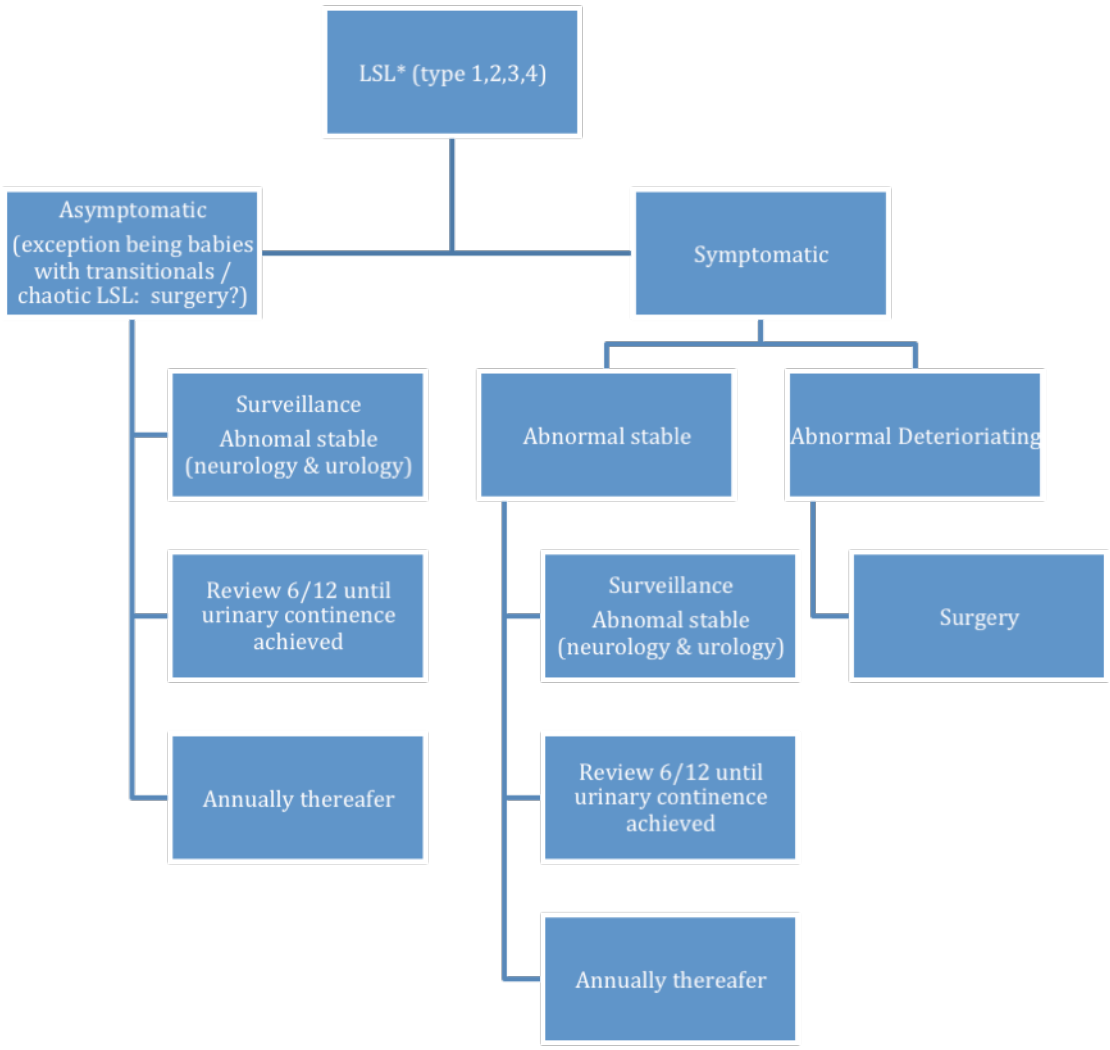


Figure 8.3 Proposed algorithm / pathway for management of LSL

8.4. A preliminary analysis of the impact of LSL on the parents of children with LSL

The impact on the parents of children with LSL has not been previously explored and this thesis has provided a preliminary description of this impact and identified gaps in our current knowledge.

The child cannot be viewed in isolation and family centred care is important in the management of a child with a chronic illness. The impact of a child's disease can affect the organisation and functioning of the family unit and in addition, can impact on the disease trajectory (Cipolletta et al., 2015).

The current study identified that:

- Parents varied in their perception of their child's needs, and this may be of importance to clinicians when deciding on a management plan, for example when assessing disease progression or patient adherence to treatment regimes (for example CIC).
- Parents of children with transitional lipomas were found to be at higher risk of stress and anxiety, and this finding suggests that support could be targeted to this group of parents.
- There was a trend for parents of children with LSL to have increased anxiety and to a lesser degree depression, when compared to parents of healthy children. Urology and pain were the only domains associated with increased anxiety and depression in parents, but deficits in neurology and urology were associated with increased stress, as was increased pain.
- Parents were anxious about future prospects for their children and about the potential for clinical deterioration.

These findings suggest the need for greater support for parents of children with LSL, in particular the transitional group who are at an increased risk of clinical deterioration.

8.5. Limitations of the study

A number of limitations need to be considered and include the following:

- The children in the current study were under the care of one institution and consequently received the same provision of health care. This resulted in a small sample size, particularly when dividing into lipoma subtypes; however, as LSL is a relatively rare and specialised condition, detailed evaluation of a small sample size still makes a valuable contribution to our knowledge and understanding.
- All children were managed by the same neurosurgeon, who provided the same management including the decision to operate and the type of

surgical technique. Urodynamic investigations and surveillance were consistent, as was physiotherapy assessment and treatment. These factors could be considered as a source of bias in outcome reporting, while a mulitcentred study design may have provided a wider and potentially more diverse sample of participants and management plans. A longitudinal study although resource intensive, would have enabled clinical changes to be assessed alongside HRQL assessment, facilitating correlation between HRQL, and clinical deterioration and age.

- The cross sectional design of the study provides a snapshot of clinical and HRQL results and does not capture variations in results or determine causality, and a longitudinal study is required to address this limitation.
- Difficulty in recalling events may have led to recall bias when respondents completed the HRQL and pain questionnaires.
- Whilst different measures were required to identify those most useful to measure the outcomes in children with LSL, the number of instruments selected presented a potential burden for respondents in particular the CHQ-PF50 and CHQ-CF87, which respondents explained was time consuming and more adapted to the American lifestyle. The absence of similar summary scores between the two CHQ questionnaires raised the question whether such analysis in a different study would produce the same components.
- Several pain measures were used in the thesis rendering analysis difficult, whereas a single measure of pain assessment would provide more consistency. The PedsQL pain questionnaire (PPQ) provides location and severity of pain and as such, would be an appropriate questionnaire to use in future studies. Participant involvement in identifying appropriate questionnaire selection in future studies should be considered and is highlighted in the recent Nuffield Council on Bioethics report Children and clinical research: ethical issues (NUFFIELD COUNCIL ON BIOETHICS, 2015)
- Although the simplicity of the G-CAT provides limitations in the amount of data provided, it's very simplicity means it may be more likely to be used in a busy neurosurgical outpatient setting. By working collaboratively with

clinicians from centres outside the study centre, the G-CAT can be assessed for reliability and validity; until that time, it cannot be the main assessment tool by which to assess for clinical deterioration in children with LSL.

8.6. Strengths of the study

There are several strengths in the current study and include the following:

- Firstly, this is the only study to undertake a systematic review of lumbosacral lipoma in children and identify the assessment methods by which to assess this rare disease. The requirement for standardised outcome measurements is being addressed by the development of an objective, standardised tool, the G-CAT.
- The study has identified specific clinical outcomes associated with LSL type; this has not been examined before and provides information regarding potential prognosis and management for the individual child.
- The study provides the first assessment of HRQL in children with LSL including qualitative assessment and an insight into how this chronic disease affects parents.
- Information from the study will lead to the publication of an information leaflet for children with LSL and their families, addressing some of the uncertainties and anxieties expressed in our discussions.

8.7. Reflection on the challenges

- As a novice researcher, undertaking the thesis has been a steep learning curve as although an experienced clinical nurse, my experience with qualitative research was limited. In retrospect, specific education regarding qualitative research would have been advantageous prior to undertaking the thesis.
- Undertaking a PhD while working full time is a major time commitment and it proved challenging at times to prioritise PhD work over clinical work: In attempting to minimise disruption for the child and family, I

discussed the questionnaires with the participants during their appointment with the urodynamics department and prior to their afternoon appointment with neurosurgery. Our meetings were regularly interrupted by the child's need to urinate following which a bladder ultrasound had to be undertaken. Thus in attempting to lessen the burden to the family, I spent extended time in the urodynamics department, and reduced my available time for clinical commitments as a consequence. This did not however affect the quality of my data collection, but suggested the need for improved time management.

- Participant recruitment presented a further challenge: I was known to many of the children and families as a clinician, and our discussions about the research study were often seen by the participants as an opportunity to ask questions related to their / their child's disease and prognosis. I was able to assist them with the majority of their questions, but separated these questions from the research agenda in an attempt to reduce potential bias in their responses to questionnaires.

8.8. Finally, as a nurse I encountered difficulties in standing back as a researcher from a group of children I cared for clinically, and this required advice and guidance from my supervisors. In addition, action research undertaken with my fellow nurse consultants, provided the education and support required for me to clinically care for the child and family, whilst undertaking research on the same client group (Gregorowski et al., 2013)

8.9. Future research

This thesis has highlighted gaps in research regarding children with LSL, including the limitations in our understanding of deficits specific to lipoma types and the burden that this chronic disease has on the children and their parents. As a result of these findings, suggestions were identified for future research and are discussed below.

There is a requirement to identify outcomes of treatment and interventions in health service provision and policy initiatives. There is also a need to identify specific environmental and social barriers this group of children may face in school and when transitioning into adulthood, with regard to opportunities and

work restrictions. A child cannot be seen in isolation, but in the context of personal factors (including the family) and the environment in which the he / she lives. School is a large part of a child's life, yet the current research has found that limitations in participation exist for some children within the school environment. Government policy must support the opportunities for children with a chronic illness to participate in school activities including sport, and aim to provide them with the same opportunities as more able bodied children. The requirement for care assistance and environmental modification for example, would help promote the participation in activities within the school environment, particularly for those children who regularly need to attend to their urinary and bowel continence. There is currently a lack of understanding and education regarding sphincter continence from professionals, peers and friends, and additional financial resources may be required to address this.

All intervention strategies planned need to be practical and achievable, both from the clinical viewpoint (maximising function and limiting disability and pain), the psychosocial viewpoint (providing appropriate interventions) and from the viewpoint of policy makers (addressing environmental barriers, encouraging participation of disabled children in activities and addressing social prejudices and inequalities).

Further areas suggested for future research are suggested in the following table:

Table 8.3 Suggested areas for future research

Influencing factor	Future research areas
Impact of disease	• Engage commissioners with care pathways specific to the needs of children with LSL, to ensure the implementation of financial and political requirements specific for the management of continence, and to identify strategies for addressing highlighted

difficulties.

- Develop further research regarding LSL type (specifically the size and location of a syrinx) and the extent of symptom occurrence/ deterioration, which would further guide the family, clinician and policy makers.
- Investigate whether sphincter innervation is more susceptible to the effects of tethering in females compared with males
- Evaluate if the use of the newly published HRQL questionnaire for children with spinabifida is appropriate for children with LSL in understanding the impact of continence issues (Velde et al., 2016)
- Develop a more effect method of pain assessment and management
- Explore more fully, what matters to children with LSL and their parents by the use of interviews
- Identify the role of ethnicity and culture in the experience of children with LSL and their parents
- Develop interventions to improve HRQL
- Integrate the framework of The International Classification of Functioning, Disability and Health for Children and Youth (ICF-CY) (World Health Organisation, 2007) into the management of children with LSL, to assess and address personal and environmental barriers to participation in activities
- Identify the economic burden of LSL with regard to co morbidities and care giver time costs

Knowledge of disease process	• Creation of an information leaflet including findings from the research and prognostic features associated with LSL type, which is available on the Internet for parents to access, particularly during the neonatal period when parents require accurate information
Emotional health	• Identify the reasons why specific emotional difficulties including reduced self-esteem occur in children with LSL and develop an appropriate intervention plan
Parental coping	• Investigate systems by which to support parents in coping with their child's disease

8.10. Conclusion

An evidence based objective clinical assessment tool (the G-CAT) has been developed for children with LSL that can be used in the outpatient setting. In addition, the appropriate parameters to be used to assess the HRQL of children with LSL have been identified. Combining the clinical and HRQL assessment tools provides the clinician with a holistic view of the current status of the individual child and can be used to determine a management plan.

The aims of the thesis have provided several original contributions to the literature and are discussed below.

This thesis has focused on a heterogeneous condition and suggested an assessment tool, the G-CAT, that incorporates clinical and HRQL assessment for this group of children regardless of LSL type, with the goal of optimising health, minimising symptomology and disability, and informing children, parents and clinicians. The aim of the G-CAT is to address the current variations in methods of assessment and management identified as important by NHS England, and provide standardised data that can guide clinicians in providing

consistent management. The G-CAT must provide simplicity of use, and provide a basis by which to audit longitudinal outcome results nationally and internationally. These outcome results can then be used to help improve the quality of care provided for a very rare condition via a collaborative approach.

The thesis has also provided evidence that can inform and guide policy makers and promote the appropriate allocation of resource provision to children with LSL. Although future research is required, findings from the thesis suggest that specific intervention strategies are required regarding the management of sphincter function, particularly within the school environment.

Furthermore, evidence of associations between LSL types, and clinical and HRQL outcomes has been addressed, which can offer a prognosis and identify interventions specific to the individual child.

The thesis has gone part way to explaining the burden that LSL puts on the individual, the family and the health care system, and emphasised the requirement for analysing and reducing heterogeneities in management and health service use.

The new understanding provided from the thesis will improve the care provided to children with LSL and their families, through the provision of increased knowledge of the disease, the use of standardised assessment and the ability for clinicians to share audit across institutions nationally and internationally.

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Appendix 1

Conference proceedings

1. **44th Annual meeting of the International Society of Pediatric Neurosurgery (ISPN).**
   October 24th 2016 (accepted for presentation)
   Kobe, Japan
   Is there concordance between child and parent ratings of Health Related Quality of Life reports, in Children with Spinal Dysraphism?

2. **25th Congress of the European Society for Pediatric Neurosurgery (ESPN)**
   May 8th 2016
   Paris
   The Health Related Quality of Life of Parents of Children with Chronic Disease.

3. **43rd Annual meeting of the International Society of Pediatric Neurosurgery (ISPN).**
   October 5th 2015
   Izmir, Turkey
   The Health Related Quality of Life of Children with Lumbosacral lipoma.

4. **Centre for Nursing and Allied Health Research and Evidence Based Practice-Orchid study day: It's the translation of research into practice that counts: examples from nurses and AHPs**
   June 19th 2015
   London
   Clinical outcomes in children with caudal, dorsal and transitional lumbosacral lipomas (poster presentation).

5. **42nd Annual Meeting of the International Society of Pediatric Neurosurgery (ISPN)**
   November 9th 2014
   Rio de Janeiro, Brazil
   Recognition of neurosurgical research in nursing practice.

Publications

MAY, L., HAYWARD, R., CHAKRABORTY, A., FRANCK, L., MANZOTTI, G., WRAY, J. & THOMPSON, D. **2013.**
Lack of uniformity in the clinical assessment of children with lipomyelomeningocele: a review of the literature and recommendations for the future. *Childs Nerv Syst.*

Appendix for chapter 2

2.1 Data extraction tool

Data extraction form
Title of review: Systematic review of LSL
PART ONE: REVIEW, REVIEWER AND STUDY INFORMATION
Study ID:
Reviewer Name
Date of completion of form
Type of article
Author(s)
Source details (year of publication, journal / other, volume, page numbers)
Language of publication
Type of report (published journal, thesis etc)
PART TWO: STUDY ELIGIBILITY
Type of study (RCT, case study etc)
Participants (Did the participant / child have LSL? Yes/ no /unclear)
Interventions
Has the child had surgery for LSL? Yes / No
Is there a comparison group in the study? Yes / No
If you have answered NO to any of the questions about participants, interventions or outcomes please STOP HERE. If there was no comparison group but you have answered YES to the other questions or if you have answered YES for all questions, please proceed to Part 3.
PART 3: INFORMATION ABOUT THE STUDY
Characteristics of the study
Country where the study was conducted
Date study undertaken (data collection)
Number of participating centres
Characteristics of the participants
Inclusion criteria (please describe)
Exclusion criteria (please describe)
Number of participants at baseline (in total)
Number of participants not followed-up
Reasons participants not followed-up
Types of comparison group
Intervention group

Duration of follow-up
Age range of participants
Age of participants (mean, S.D.)
Age range of children if participants are parents
Gender – number % female
Gender – number % male
Ethnicity of participants
Socioeconomic status of participants
Disease characteristics
Type of spinal lipoma
Previous surgery
Time since surgery
Co-morbidities
Types of outcomes
Primary outcome
What was / were the primary outcome(s)?
How was the primary outcome assessed (questionnaire, observation etc)
Who completed the primary outcome measure (participants, observer)
Time between baseline and follow-up
Were any adverse events reported?
PART FOUR: STUDY QUALITY
How were the patients selected? (convenience sample, all patient from data base included etc
Method of analysis (per protocol, intention to treat)
Were the study groups comparable at baseline (list factors on which groups were compared
Number of participants lost to follow-up (give numbers overall and for each group and reasons for attrition
For non-randomised designs, on what factors were groups compared at baseline and were they comparable?
For all studies: Were hypotheses stated prior to the start of the study? Yes / No Were all aspects of the study conducted prospectively? Yes / No Was the intervention comprehensively described and replicable? Yes / No Was training for the delivery of the intervention described? Yes / No Were validated measures used for outcome assessment? Yes / NO Were confounding factors considered? Yes / No . If so, which?

What methods were used to control for any confounding factors? In comparative studies, were participants seen within the same time frame within each group? Yes / No Was the fate of all patients enrolled in the study adequately described? Yes / No
Is further information required from the authors? Yes / No

2.2 Quality assessment checklist

Quality domain	Coding	Explanation of quality criteria
Sample	A: Adequate I: Inadequate U: Unclear/unknown	Adequate: • Eligibility criteria explained and sample includes children with LSL. • Sampling methods detailed and appropriate to study. • Explanations of exclusions, refusals and withdrawals. Inadequate: • Inappropriate sampling for study e.g. sample selected by convenience • Sample size too small for study Unclear: • Sample selection (eligibility, recruitment methods, size) was not explained. • Sample does not include children with LSL • Exclusions, refusals and withdrawals not detailed.
Outcomes/main Measures/ tools of assessment used	A: Adequate I: Inadequate Unclear/unknown	• Details provided of how (any) bias was addressed • Study explained and appropriate, intended outcomes addressed • Details of outcome measurement /tools of

		assessment used for children with LSL. • Evidence of internal consistency, reliability and validity. • Inadequate • Not appropriate to study population and / or design. • Outcome measure not tested or reliable/valid. Unclear • Not all results given. • Unclear/absent outcomes / measures /tools of assessment of symptoms
Decision for Inclusion in review	Inc.: include Inc*: include but interpret results with caution Exc: exclude from review	Inc • All criterion rated adequate Inc* • One criterion adequate, two unclear • One criterion adequate, one unclear, one inadequate • Two criterion adequate, one unclear • Two criterion adequate, one inadequate • Three criterion unclear Exc • Two or more criterion inadequate

Appendix for chapter 3

3.1 Research and Development approval, UCL Institute of Child Health, London

UCL INSTITUTE OF CHILD HEALTH

Great Ormond Street Hospital for Children NHS Foundation Trust

Joint Research and Development Office
Division of Research and Innovation

Direct Line: 020 7905 2698
Email: Marice.Lunny@gosh.nhs.uk

08/01/2013

Mrs Lindy May
Nurse Consultant, Neurosurgery
Great Ormond Street Hospital
Great Ormond Street
London

Dear Mrs Lindy May

PROJECT TITLE	Understanding the quality of life issues associated with lipomyelomeningocele, a type of spina bifida occulta
Protocol version	Version 4
Protocol date	03 September 2012
REC Reference	12/LO/1574
R&D Reference	11NR48
CSP Reference	N/A
Sponsor	GOSH
Chief Investigator (CI)	Mrs Lindy May

Notification of Great Ormond Street Hospital NHS Permission.

The research approval process for the above named study has been completed successfully. I am pleased to issue approval on behalf of Great Ormond Street Hospital for Children NHS Trust (GOSH) for the above study to proceed.

All research carried out within this Trust must be in accordance with the principles set out in the Research Governance Framework for Health and Social Care (April 2005, 2nd edition, Department of Health (DoH)).

This approval is issued on the basis of the project documentation submitted to date. The approval may be invalidated in the event that the terms and conditions of any research contract or agreement change significantly and while the new contract/agreement is negotiated.

The conditions for host site approval are as follows:

- The Principle Investigator (PI) must ensure compliance with protocol and advise the Joint R&D Office of any change(s) to the protocol. Failure of notification may affect host approval status.
- Under the terms of the Research Governance Framework (RGF), the PI is obliged to report any Serious Adverse Events (SAEs) to the Sponsor and the Joint R&D Office in line with the study

protocol and Sponsor requirements. Adverse Incidents (AEs) must also be reported in accordance with the Trust Adverse Incident Reporting Policy & Procedures.

- The PI must ensure appropriate procedures are in place to action urgent safety measures.
- The PI is responsible for the set up and maintenance of the Investigator Site File (ISF) generated to store all documentation relating to this project.
- The PI must ensure that all named staff are compliant with the Data Protection Act (DPA) 1998, Human Tissue Act (HTA) 2005, Mental Capacity Act (MCA) 2005 and all other applicable statutory guidance and legislation.
- The PI must allow monitoring and auditing by the Sponsor and the Joint R&D Office.
- The PI must report any cases of suspected research misconduct and fraud to the Joint R&D Office.
- The PI must provide an annual report to the Joint R&D Office for all research involving NHS patients, staff and/or resources. The PI must notify the Joint R&D Office of any presentations of such research at scientific or professional meetings, or on the event of papers being published and any direct or indirect impacts on patient care.

Failure to comply with the above conditions and regulations will result in the suspension of the research project.

Please contact the Joint R&D Office if you require any further guidance or information on any matter mentioned above. We wish you every success in your research.

Yours sincerely,

[redacted]

Marice Lunny
Senior Research Governance Manager
Joint Research and Development Office

3.2 Research Ethics Committee approval, Chelsea, London

NHS
Health Research Authority

NRES Committee London - Chelsea
HRA
Research Ethics Committee (REC) London Centre
Ground Floor
80 Skipton House
London Road
London, SE1 6LH

Telephone: 020 7972 2556

10 December 2012

Mrs Lindy May
Nurse Consultant
Great Ormond Street Hospital for Children NHS Foundation Trust
Koala ward, Great Ormond Street Hospital, Great Ormond Street, London
WC1N3JH

Dear Mrs May

Study title:	**Functional Status and Health Related Quality of Life of Children and Adolescents with Spinal Lipoma**
REC reference:	**12/LO/1574**
IRAS project ID:	**110456**

Thank you for your letter of 12 November 2012, responding to the Committee's request for further information on the above research and submitting revised documentation.

The further information has been considered on behalf of the Committee by the Chair.

We plan to publish your research summary wording for the above study on the NRES website, together with your contact details, unless you expressly withhold permission to do so. Publication will be no earlier than three months from the date of this favourable opinion letter. Should you wish to provide a substitute contact point, require further information, or wish to withhold permission to publish, please contact the Co-ordinator Mr Thomas McQuillan, thomas.mcquillan@nhs.net.

Confirmation of ethical opinion

On behalf of the Committee, I am pleased to confirm a favourable ethical opinion for the above research on the basis described in the application form, protocol and supporting documentation as revised, subject to the conditions specified below.

Ethical review of research sites

NHS sites

The favourable opinion applies to all NHS sites taking part in the study, subject to management

Other: Table 3: Questionnaires/Assessments		
Other: List of other issues that may be related to the disease (Spinal Lipoma)n		12 November 2012
Participant Consent Form: For Children (Aged 4 - 7 Years Old)	4	12 November 2012
Participant Consent Form: For Children (Aged 8 - 12 Years Old)	4	12 November 2012
Participant Consent Form: Young Person	4	12 November 2012
Participant Consent Form: Parent	4	12 November 2012
Participant Consent Form: Parent Consent for Child	4	12 November 2012
Participant Information Sheet: For Children (Aged 4 - 7 Years Old)	4	12 November 2012
Participant Information Sheet: For Children (Aged 8 - 12 Years Old)	4	12 November 2012
Participant Information Sheet: For Children (Aged 13 - 18 Years Old)	4	12 November 2012
Participant Information Sheet: Parents and Carers	4	12 November 2012
Protocol	4	03 September 2012
Questionnaire: NEM Scale		
Questionnaire: PedsQL pain score		
Questionnaire: PAQ		
Questionnaire: PedsQL		
Questionnaire: CHQ		
Questionnaire: Pierrs-Harris 2 (PH2)		
Questionnaire: Paediatric Inventory for Patients (PIP)		
Questionnaire: SF36		
Questionnaire: Hospital Inventory for Parents (PIP)		
REC application	110456/359899/1/972	03 September 2012
Response to Request for Further Information		12 November 2012
Summary/Synopsis		

Statement of compliance

The Committee is constituted in accordance with the Governance Arrangements for Research Ethics Committees and complies fully with the Standard Operating Procedures for Research Ethics Committees in the UK.

After ethical review

Reporting requirements

The attached document *"After ethical review – guidance for researchers"* gives detailed guidance on reporting requirements for studies with a favourable opinion, including:

- Notifying substantial amendments
- Adding new sites and investigators
- Notification of serious breaches of the protocol
- Progress and safety reports
- Notifying the end of the study

The NRES website also provides guidance on these topics, which is updated in the light of changes in reporting requirements or procedures.

Feedback

You are invited to give your view of the service that you have received from the National Research Ethics Service and the application procedure. If you wish to make your views known please use the feedback form available on the website.

Further information is available at National Research Ethics Service website > After Review

12/LO/1574	**Please quote this number on all correspondence**

We are pleased to welcome researchers and R & D staff at our NRES committee members' training days – see details at http://www.hra.nhs.uk/hra-training/

With the Committee's best wishes for the success of this project.

Yours sincerely

[redacted]

Dr Shelley Dolan
Chair
Email:NRESCommittee.London-Chelsea@nhs.net

Enclosures: "After ethical review – guidance for researchers" *[SL-AR2]*

Copy to: *Miss Marice Lunny, Great Ormond Street Hospital for Children NHS Foundation Trust*

3.3 Child and parent letters of invitation and information sheets

Great Ormond Street Hospital for Children NHS Foundation Trust

Parent's information sheet

Functional Status and Health Related Quality of Life of Children and Adolescents with Spinal Lipoma

Invitation to take part in a research study

Dear Mr and Mrs ….,

I am undertaking a research study to find out what it is like to live with a spinal condition known as a spinal lipoma. As your child was born with this condition, I am inviting both you and your child to take part in the study. This information sheet tells you what will happen if you and your child agree to take part. It is entirely up to you all to decide if you want to take part and your child's care at the hospital will not be affected if you decide not to be involved. I will not ask your child to take part unless you have said it is OK to ask him/her.

Why have I been approached?

All children with spinal lipoma under the care of the hospital and their parents will be asked to take part in the study. I am asking your permission to discuss the study with your child, so please read on to see what is involved.

Who is doing the study?

I am doing this study as part of a Doctor of Philosophy degree (PhD) at University College, London (UCL). Mr Thomson (your neurosurgeon) and Lucy Alderson (the physiotherapist) will also help with the study.

What's the purpose of the study?

Spinal lipoma is a rare condition with each paediatric neurosurgical centre treating relatively small numbers of children. We know that urine and bowel function can be affected in some children, and as they grow up some children find it more difficult to join in sports at school or be as active as their friends due to difficulty with mobility or pain. This certainly doesn't happen to all children with spinal lipoma and because each child is individual, would like to understand more about the physical affect this has on your child and the emotional affect it may have on both you and your child. This information will help us understand what care and support you and your child may need from the hospital and the community.

What will happen during the study?

Your child will attend his / her normal outpatient appointments with your neurosurgeon. During this time your child will be examined by the Doctor and physiotherapist as usual.

- The physiotherapist will ask your child to undertake some extra simple exercises (which takes about 15 minutes) so we can understand what affect the spinal lipoma may have on your child's physical activities.

- Both you and your child will be asked to complete some questionnaires (which takes about 40 minutes) which will provide us with further information about the physical affect spinal lipoma may have on your child's daily life and also about any emotional affect.
-

By asking you to attend your appointment slightly early, I aim to reduce any inconvenience to you as much as possible. Tea/ coffee/cordial/water will be provided and toilet facilities are available. It may be more convenient for you to complete questionnaires /interview when you attend the hospital for your routine appointment with the Urodynamics team. If time is difficult for you then, I can with your permission, arrange to visit you at your home to undertake the interview.

Basic information about your child will be collected from the medical notes (age, any operations or treatments).

Do we have to take part?

Neither you nor your child has to take part and if you do take part, you can withdraw from the study at any time should you so choose. A decision to not take part or to withdraw will not affect your child's care or the standard of care he / she receives.

How will the information be kept?

- In accordance with the UK's Data Protections Act 1998, data collected regarding you and your child will be kept confidential and secure and used only for the purpose for which it is collected.
- The tape recorded interviews will be copied onto a computer and the tapes erased once this is completed.
- Data on a computer will be password protected
- Information obtained from yourself and your child will be kept by the Principal Investigator for *one year after completion of the study* after which time it will be deleted / shredded.
- All data is anonyms- children and their parents will be given a study number, which will only be known to me.
- Only I will have access to the information although I may show this to my PhD supervisor.
-

Are there any risks to me or my child and what do I do if I am worried about the study?

We do not anticipate there to be any risks in taking part in the study. However, we do know that it can sometimes be upsetting to talk about your child's health. If either you or your child becomes upset whilst answering the questions we will stop. If you or your child wants to be referred to someone in the clinical team who can help you I will arrange this for you.

What are the possible benefits for me and / or my child taking part?

Taking part may not have a direct effect on you or your child although you may find it helpful to talk about things.

What happens if I am worried about the study?

If you have any questions about the study you can contact me on 02077626753 and I will do my best to answer them. If you remain unhappy with my answer or want to complain formally you can contact the PALS office.

Who is supporting the study?

The study is supported by the neurosurgical team at the hospital and my PhD supervisor.

All research in the NHS is assessed by an independent group called the Research Ethics Committee. This study has been assessed by …..who have given it a favourable opinion. Research Ethics Committees are involved to ensure the dignity, wellbeing, safety and rights of both you and your child are maintained throughout the study.

Payment.

Neither you nor your child will be paid for taking part in the study. However, drinks and snacks will be available.

What happens now?

All families whose child is under the care of Mr Thompson at the hospital are being sent this information leaflet and asked to respond to a research assistant - Louise Bradshaw, by e mail, phone or using the reply slip enclosed. If you and your child agree to take part, you will be asked if it is convenient to arrive early for your appointment with the neurosurgeon, whether you prefer to complete the study when you attend your appointment with the Urodynamics team, or if is more convenient to for me to visit your home for the interview section of the study. As the Principal Investigator, I will meet you when you come to clinic, to discuss the study further , answer any questions you may have and take consent from you and your child (your child will be provided with a consent form to sign himself / herself if he / she is over 16 years of age, or an assent form if he / she is under 16 years of age) .

If you do decide to take part, I will inform your GP so that he / she is aware of the study and has my contact details should any further information be required.

Thank you for taking time to read this information sheet.

Yours Sincerely

Lindy May

Nurse consultant, neurosurgery

Further Information. Please phone Lindy May if you require further information; Telephone: 0207 762676. E mail : Lindy. May@gosh.nhs.uk. Research assistant: Louise Bradshaw; Telephone 02074059200 ext 5833; e mail: Louise.Bradshaw@gosh.nhs.uk

Great Ormond Street Hospital for Children NHS Foundation Trust

Information sheet for young people (13-18 years old)

Dear (insert name),

I would like to invite you to take part in a research study when you attend an outpatient appointment at The Hospital for Children, Great Ormond Street.

The study is being undertaken by Lindy May, nurse consultant in neurosurgery at the hospital. It is important you understand why the study is being done and what is involved. This information sheet provides details about the study to help you decide if you would like to take part. The decision is entirely up to you and if you choose to be in the study or not, your normal care will not be affected.

What is research?

Research is a way we try to find out answers to questions and in addition, what's important to you.

Why Have I been asked if I want to take part?

I am hoping to study as many children and young people as possible who were born with a spinal (back) lump such as you had, which is called a spinal lipoma. I would also like to involve parents. Please read on to see what the study involves. You can discuss it further with me, your GP, your family or any of the doctors or nurses if you want to.

Who is doing the study?

I am doing this study as part of a Doctor of Philosophy degree (PhD) at University College, London (UCL). Mr Thomson and Lucy Alderson (your physiotherapist) will also help with some parts of the study.

What's the purpose of the study?

Spinal lipoma is very rare and we would like to know if this has any effect on your day to day life. *The effect that a spinal lipoma has on children and young people varies hugely between individuals*. This information will help us understand what help and support we may be able to offer you and other children and young people with the same condition. I will also be talking to your parents too, if that's OK

What will happen during the study?

When you have your routine out -patient appointment with Mr Thompson, he and the physiotherapist will examine you as usual. In addition,.

- The physiotherapist will ask you to walk for 6 minutes, to see how far you can walk during that time. She will also analyse your gait (how you walk) to help us understand any difficulties young people who were born with spinal lipoma, may have with walking. This only takes about 5 minutes and you will be asked to walk on a special “walkway” which is hooked up to a computer which analyses the information.
- You will be asked to complete some questionnaires (which would take at most, 40 minutes) to help us understand you better. Some of them will be about physical things such as sporting activities and others will be about emotional things and how you are feeling.
- I may ask you to keep a pain diary- which helps us understand if and when any pain you have might be worse, and what makes it worse. I will explain to you how to fill it in!

By asking you to attend your appointment slightly early, I aim to reduce any inconvenience to you as much as possible. It may be more convenient for you to complete questionnaires /interview when you attend the hospital for your routine appointment with the Urodynamics team. If necessary, I can ask you and your parent’s permission if it’s Ok to visit you at home at a convenient time to do the interview.

Basic information about you will be collected from the medical notes (age, any operations or treatments).

Do I have to take part?

No, you do not have to take part and even if you do, you can withdraw from the study at any time should you so choose. A decision to not take part or to withdraw will not affect your care and treatment.

Will anyone else know I am doing this?

If you decide to take part in the study, we will keep any information you give us private. No one will know except you, your parents and anyone you chose to tell yourself !

How will the information be kept?

- In accordance with the UK’s Data Protections Act 1998, data collected will be kept confidential and secure and used only for the purpose for which it is collected.
- The tape recorded interviews will be copied onto a computer and the tapes erased once this is completed.
- Data on a computer will be password protected.
- Information obtained will be kept by the Principal Investigator for *one year after completion of the study* after which time it will be deleted / shredded.
- All data is anonyms- everyone who is in the study is given a study number, which will only be known to me.
- Only I will have access to the information although I may show this to my PhD supervisor.

If you tell me anything that means you are at risk of harm, danger or injury I will have to deal with this to make sure you are safe. This might mean telling you parents if necessary.

Are there any risks and what happens if I am worried about the study?

It is not thought that there will be any side effects or risks from taking part in the study but should you want to discuss any issues that might worry you, please talk to your parents, friends, myself or Mr Thompson. If you want to discuss the study before deciding whether you want to take part, do please phone me on 02077626753 so we chat about it. If you are still concerned or worried we can refer you for further advice or support if needed.

What are the possible benefits for me?

Taking part may not have a direct effect on you but we hope to get a better understanding how the problem with your back might affect your life and what's important to you and your family.

Who is supporting the study?

The study is supported by the neurosurgical team at the hospital, and my PhD supervisor.

All research in the NHS is assessed by an independent group called the Research Ethics Committee. This study has been assessed by …..who have given it a favourable opinion. Research Ethics Committees are involved to ensure your rights, dignity, wellbeing and safety are maintained throughout the study.

Payment.

There is no payment for taking part in the study but I will make sure you get drinks and snacks!

What happens now?

Your parents have been sent information about the study and asked if they are happy to take part and if they are happy for you to be asked if you want to take part. They must have said it was Ok because I have now sent you this information leaflet to read!

If you have decided to take part, when you arrive at the clinic both you and your parents will be given the opportunity to ask any questions and then asked to sign an agreement form. I will give you a copy of the form to keep.

Thank you for taking the time to read this letter.

Lindy May

Nurse consultant, neurosurgery

Tel: 0207 6726753 ; Lindy.May@gosh.nhs.uk

Information sheet for children (8-12 years old)

Living with spinal lipoma (that's the problem with your back you were born with)

Dear …….

Your parent(s) have said I can talk to you about being in a study about what it's like living with a spinal lipoma- which is the problem with your back you were born with. This information sheet tells you what will happen if you agree to take part.

You can decide yourself if you want to take part and your normal treatment will not change whatever you decide.

Please ask if you have any questions about the study

What is research?

Research is a way to try to find answers to questions and also, to find out what's important to you.

Why Have I been asked if I want to take part?

I am hoping to study as many children and young people as possible who were born with a spinal (back) lump such as you had, which is called a spinal lipoma. I would also like to involve parents. Please read on to see what the study involves. You can discuss it further with me, your GP, your family or any of the doctors or nurses if you want to.

Who is doing it?

I am doing the study as part of a degree course (called a PhD) at University. Mr Thompson, your neurosurgeon, knows all about the study. Mr Thomson and Lucy Alderson (your physiotherapist) will also help with some parts of the study.

Why is the study being done?

I'm interested in talking to children like you and to understand more about your day to day life. I will also ask your *parent(s)* what they think too, if that's OK.

This information will help us understand what help and support we may be able to offer you and other children and young people with the same condition.

What will happen during the study?

When you have your routine out -patient appointment with Mr Thompson, he and the physiotherapist will examine you as usual. In addition:

- The physiotherapist will ask you to walk for 6 minutes, to see how far you can walk during that time. She will also look at how you walk to help us understand any difficulties young people who were born with spinal lipoma, may have with walking. This only takes about 5 minutes and you will be asked to walk on a special “walkway” which is hooked up to a computer which analyses the information.
- You will be asked to complete some questionnaires (which would take at most, 40 minutes) to help us understand you better. Some of them will be about physical things such as sporting activities and others will be about emotional things and how you are feeling..
- I may ask you to keep a pain diary- which helps us understand if and when any pain you have might be worse, and what makes it worse. I will explain to you how to fill it in!

By asking you to attend your appointment slightly early, I aim to reduce any inconvenience to you as much as possible. It may be more convenient for you to complete questionnaires /interview when you attend the hospital for your routine appointment with the Urodynamics team. If necessary, I can ask you and your parent’s permission if it’s Ok to visit you at home at a convenient time to do the interview. Basic information about you will be collected from the medical notes (age, any operations or treatments).

Do I have to take part?

No- it’s up to you. You can decide not to take part, or change your mind- it won’t change how we look after you.

Will anyone else know I am doing this?

If you decide to take part in the study, no one needs to know except your Mum, Dad and me. You can tell anyone else if you want to!

How will the information be kept?

- You will be given a study number, which only I know.
- Any information I get (your questionnaires and the information the physiotherapist finds out about your walking for example) will be looked at, then stored on a computer, which has a password, so only I can access it. A year after the study is finished, all the information is deleted
- Papers such as questionnaires are locked in a cupboard and shredded *a year after the study is finished.*
- The tape recorded interviews will be copied onto a computer and the tapes then wiped clean.
- Data on a computer will be password protected.
- Only I have access to the information although I may show it to my supervisor.

If you tell me anything that means you are at risk of harm, danger or injury I will have to deal with this to make sure you are safe. This might mean telling you parents if necessary.

Are there any risks and what happens if I am worried about the study?

It is not thought that there will be any side effects or risks from taking part in the study but should you want to talk about anything that's worrying you, talk to your parents, your friends, me or Mr Thompson. If you are still worried we can ask someone else in the hospital to chat to you to see if we can help sort out what's worrying you.

What's in it for me?!

Taking part may not make any difference to you but by understanding what's important to children like you, we can plan to help you better.

Who's supporting the study?

The study is supported by the neurosurgical team at the hospital, and Mr Thompson knows all about it. He and Jo Wray (my supervisor) are checking how I do the study.

Studies in hospitals are checked by a group of people (called the Research Ethics Committee) to make sure you are looked after properly during the study. This study has been looked at by ……who have said it's Ok to go ahead with the study.

Payment

There's no payment for taking part, but I will provide some drinks and snacks!

What happens now?

If you want to take part in the study, tell your parents, and me!

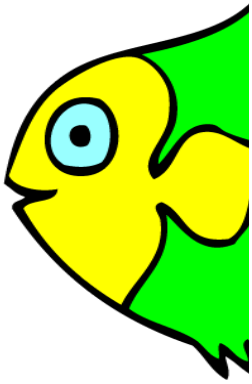
I will talk to you about the study when you come to see Mr Thompson, answer any of your questions and ask you to sign a form if you are happy to take part. Your parents will also be asked to sign a form to say they are happy for you to do this. I will give you a copy of the form to keep.

Thank you for taking the time to read this letter.

Lindy May, Nurse consultant, neurosurgery. Tel: 0207 6726753

Great Ormond Street Hospital for Children NHS

NHS Foundation Trust



Information sheet for children (aged 4-7 years old)

Hello!

I am doing a study about children who were born with a lump on their back, as you were, and I'd love to find out more about you!

This paper tells you what will happen if you want to take part- you can ask a grown up to help you read it if you want to.

If you decide to say no, we will still look after you at the hospital.

What is a study?

A study is what we do when we want to learn more about something.

If I know a little bit more about children like you, I can tell other doctors, nurses and other people looking after you what's important about you.

Why have you asked me?

Mr Thompson (your doctor here) looks after lots of children's backs and I am asking all of you to help me with my study.

Who is doing it?

I am doing most of it but Mr Thompson will be seeing you as usual, and so will Lucy, the lady who makes you do those exercises!

Why are you doing it?

If we can understand more about you, maybe we can help you more!

What will I have to do?

Mr Thompson (your doctor) will see you as normal.

Lucy (the lady that makes you do those exercises!) will ask you to do some running and walking when you come to the hospital! I will ask you to look at some questions and see if you can answer them for me..

Do I have to do it?

No you can say no. We will still look after you the same.

Will anyone else know I am doing this?

If you decide to take part in the study, no one needs to know except your *parents* and me. You can tell anyone else if you want to!

Where do you keep things you write about me?

I keep everything in a safe place so only I can see them (and the lady who makes sure I am doing everything properly)

What if I don't like it?

You must tell us or your *parents* and we can talk to you, or stop the study if you want. We will still look after you!

What happens now?

If you want to join in the study, tell your *parents* so I can come and see you in clinic.

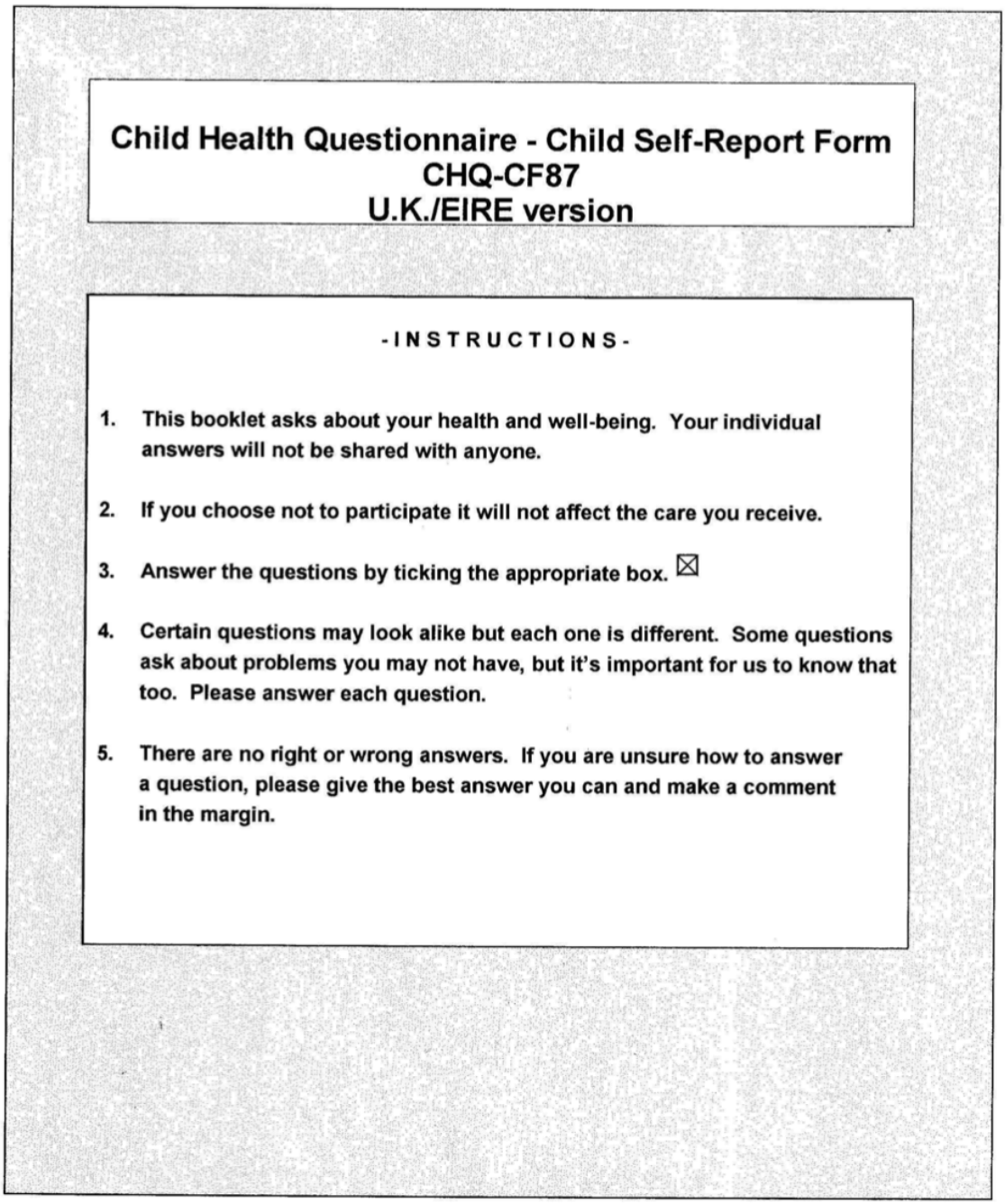
Thanks!

Lindy

3.4 Questionnaires

3.4.1 Child Health Questionnaire (CHQ)

3.4.1.1. CHQ-CF87



Child Health Questionnaire - Child Self-Report Form
CHQ-CF87
U.K./EIRE version

-INSTRUCTIONS-

1. **This booklet asks about your health and well-being. Your individual answers will not be shared with anyone.**
2. **If you choose not to participate it will not affect the care you receive.**
3. **Answer the questions by ticking the appropriate box. ☒**
4. **Certain questions may look alike but each one is different. Some questions ask about problems you may not have, but it's important for us to know that too. Please answer each question.**
5. **There are no right or wrong answers. If you are unsure how to answer a question, please give the best answer you can and make a comment in the margin.**

SECTION #1: YOUR GLOBAL HEALTH☐

1.1. In general, would you say your health is:

☐	☐	☐	☐	☐
Excellent	Very good	Good	Fair	Poor

SECTION #2: YOUR PHYSICAL ACTIVITIES

The following questions ask about physical activities you might do during a day.

2.1. During the past 4 weeks, has it been difficult for you to do the following activities due to health problems?

		Yes, very difficult	Yes, somewhat difficult	Yes, a little difficult	No, not difficult
a.	do things that take **a lot** of energy, such as playing soccer, running or hiking?	☐	☐	☐	☐
b.	do things that take **some** energy such as riding a bike or skating?	☐	☐	☐	☐
c.	walk more than 10 metres or climb several flights of stairs?	☐	☐	☐	☐
d.	get around your school, neighbourhood, or playground?	☐	☐	☐	☐
e.	walk 10 metres or climb one flight of stairs?	☐	☐	☐	☐
f.	do your tasks around the house?	☐	☐	☐	☐
g.	bend, lift, or stoop?	☐	☐	☐	☐
h.	eat, dress, bath, or go to the toilet by yourself?	☐	☐	☐	☐
i.	get in and out of bed?	☐	☐	☐	☐

SECTION #3: YOUR EVERYDAY ACTIVITIES

3.1. During the past 4 weeks, has it been difficult to do your school work or usual activities with friends because of problems like FEELING SAD OR WORRIED?

Has it been difficult to:	Yes, very difficult	Yes, somewhat difficult	Yes, a little difficult	No, not difficult
a. do certain KINDS of schoolwork or activities with friends?	☐	☐	☐	☐
b. spend the usual AMOUNT of time on schoolwork or activities with friends?	☐	☐	☐	☐
c get schoolwork DONE at all or do any activities with friends?	☐	☐	☐	☐

3.2. During the past 4 weeks, has it been difficult to do your school work or usual activities with friends because of problems with your BEHAVIOUR?

Has it been difficult to:	Yes, very difficult	Yes, somewhat difficult	Yes, a little difficult	No, not difficult
a. do certain KINDS of schoolwork or activities with friends?	☐	☐	☐	☐
b. spend the usual AMOUNT of time on schoolwork or activities with friends?	☐	☐	☐	☐
c. get schoolwork DONE at all or do any activities with friends?	☐	☐	☐	☐

3.3. During the past 4 weeks, has it been difficult to do your school work or usual activities with friends because of problems with your PHYSICAL health?

Has it been difficult to:	Yes, very difficult	Yes, somewhat difficult	Yes, a little difficult	No, not difficult
a. do certain KINDS of schoolwork or activities with friends?	☐	☐	☐	☐
b. spend the usual AMOUNT of time on schoolwork or activities with friends?	☐	☐	☐	☐
c. get schoolwork DONE at all or do any activities with friends?	☐	☐	☐	☐

SECTION #4: PAIN

4.1. During the past 4 weeks, how much bodily pain or discomfort have you had?

☐ None	☐ Very mild	☐ Mild	☐ Moderate	☐ Severe	☐ Very severe

4.2. During the past 4 weeks, how often have you had bodily pain or discomfort?

☐ None of the time	☐ Once or twice	☐ A few times	☐ Fairly often	☐ Very often	☐ Every day or almost every day

SECTION #5: GETTING ALONG

Below is a list of items that describe children's behaviour or problems they sometimes have.

5.1. During the past 4 weeks how often did each of the following statements describe you?

	Very Often	Fairly Often	Some-times	Almost Never	Never
a. acted too young for your age?	☐	☐	☐	☐	☐
b. argued?	☐	☐	☐	☐	☐
c. had a hard time paying attention?	☐	☐	☐	☐	☐
d. did not do what your teacher or parent asked you to do?	☐	☐	☐	☐	☐
e. wanted to be alone?	☐	☐	☐	☐	☐
f. lied or cheated?	☐	☐	☐	☐	☐
g. had a hard time getting others to like you?	☐	☐	☐	☐	☐
h. felt clumsy?	☐	☐	☐	☐	☐
i. ran away from home?	☐	☐	☐	☐	☐
j. had speech problems (e.g., stuttering)?	☐	☐	☐	☐	☐
k. stole things at home?	☐	☐	☐	☐	☐
l. stole things outside home?	☐	☐	☐	☐	☐
m. acted mean or moody if you did not get what you wanted?	☐	☐	☐	☐	☐
n. got really mad when you did not get what you wanted?	☐	☐	☐	☐	☐
o. found it hard to be with others?	☐	☐	☐	☐	☐
p. had a hard time getting along with others?	☐	☐	☐	☐	☐

5.2. Compared to other children your age, in general would you say your behaviour is:

☐	☐	☐	☐	☐
Excellent	Very good	Good	Fair	Poor

SECTION #6: GENERAL WELL-BEING

The following phrases are about children's moods and feelings they may have.

6.1. During the past 4 weeks, how much of the time did you:

	All of the time	Most of the time	Some of the time	A little of the time	None of the time
a. feel sad?	☐	☐	☐	☐	☐
b. feel like crying?	☐	☐	☐	☐	☐
c. feel afraid or scared?	☐	☐	☐	☐	☐
d. worry about things?	☐	☐	☐	☐	☐
e. feel lonely?	☐	☐	☐	☐	☐
f. feel unhappy?	☐	☐	☐	☐	☐
g. feel nervous?	☐	☐	☐	☐	☐
h. feel bothered or upset?	☐	☐	☐	☐	☐
i. feel happy?	☐	☐	☐	☐	☐
j. feel cheerful?	☐	☐	☐	☐	☐
k. enjoy the things you do?	☐	☐	☐	☐	☐
l. have fun?	☐	☐	☐	☐	☐
m. feel jittery or restless?	☐	☐	☐	☐	☐
n. have trouble sleeping?	☐	☐	☐	☐	☐
o. have headaches?	☐	☐	☐	☐	☐
p. like yourself?	☐	☐	☐	☐	☐

SECTION #7: SELF-ESTEEM

How do you feel about yourself, school, and others? It may be helpful if you keep in mind how other children your age might feel about these areas.

7.1. During the past 4 weeks, how good or bad have you felt about:

	Very good	Somewhat good	Neither good nor bad	Somewhat badly	Very badly
a. yourself?	☐	☐	☐	☐	☐
b. your school work?	☐	☐	☐	☐	☐
c. your ability to play sports?	☐	☐	☐	☐	☐
d. your friendships?	☐	☐	☐	☐	☐
e. the things you CAN do?	☐	☐	☐	☐	☐
f. the way you get along with others?	☐	☐	☐	☐	☐
g. your body and your looks?	☐	☐	☐	☐	☐
h. the way you seem to feel most of the time?	☐	☐	☐	☐	☐
i. the way you get along with your family?	☐	☐	☐	☐	☐
j. the way life seems to be for you?	☐	☐	☐	☐	☐
k. your ability to be a friend to others?	☐	☐	☐	☐	☐
l. the way others seem to feel about you?	☐	☐	☐	☐	☐
m. your ability to talk with others?	☐	☐	☐	☐	☐
n. your health in general?	☐	☐	☐	☐	☐

SECTION #8: YOUR HEALTH

The following statements are about health in general.

8.1. How true or false is the statement for you?

	Definitely True	Mostly True	Don't Know	Mostly False	Definitely False
a. My health is excellent.	☐	☐	☐	☐	☐
b. I was so sick once I thought I might die.	☐	☐	☐	☐	☐
c. I do not seem to get very sick.	☐	☐	☐	☐	☐
d. I seem to be less healthy than other kids I know.	☐	☐	☐	☐	☐
e. I have never been very, very sick.	☐	☐	☐	☐	☐
f. I always seem to get sick.	☐	☐	☐	☐	☐
g. I think I will be less healthy when I get older.	☐	☐	☐	☐	☐
h. I think I will be very healthy when I get older.	☐	☐	☐	☐	☐
i. I never worry about my health.	☐	☐	☐	☐	☐
j. I think I am healthy now.	☐	☐	☐	☐	☐
k. I think I worry about my health more than other kids my age.	☐	☐	☐	☐	☐

8.2. Compared to one year ago, how would you rate your health now:

☐	☐	☐	☐	☐
Much better now than 1 year ago	Somewhat better now than 1 year ago	About the same now as 1 year ago	Somewhat worse now than 1 year ago	Much worse now than 1 year ago

SECTION #9: YOU AND YOUR FAMILY

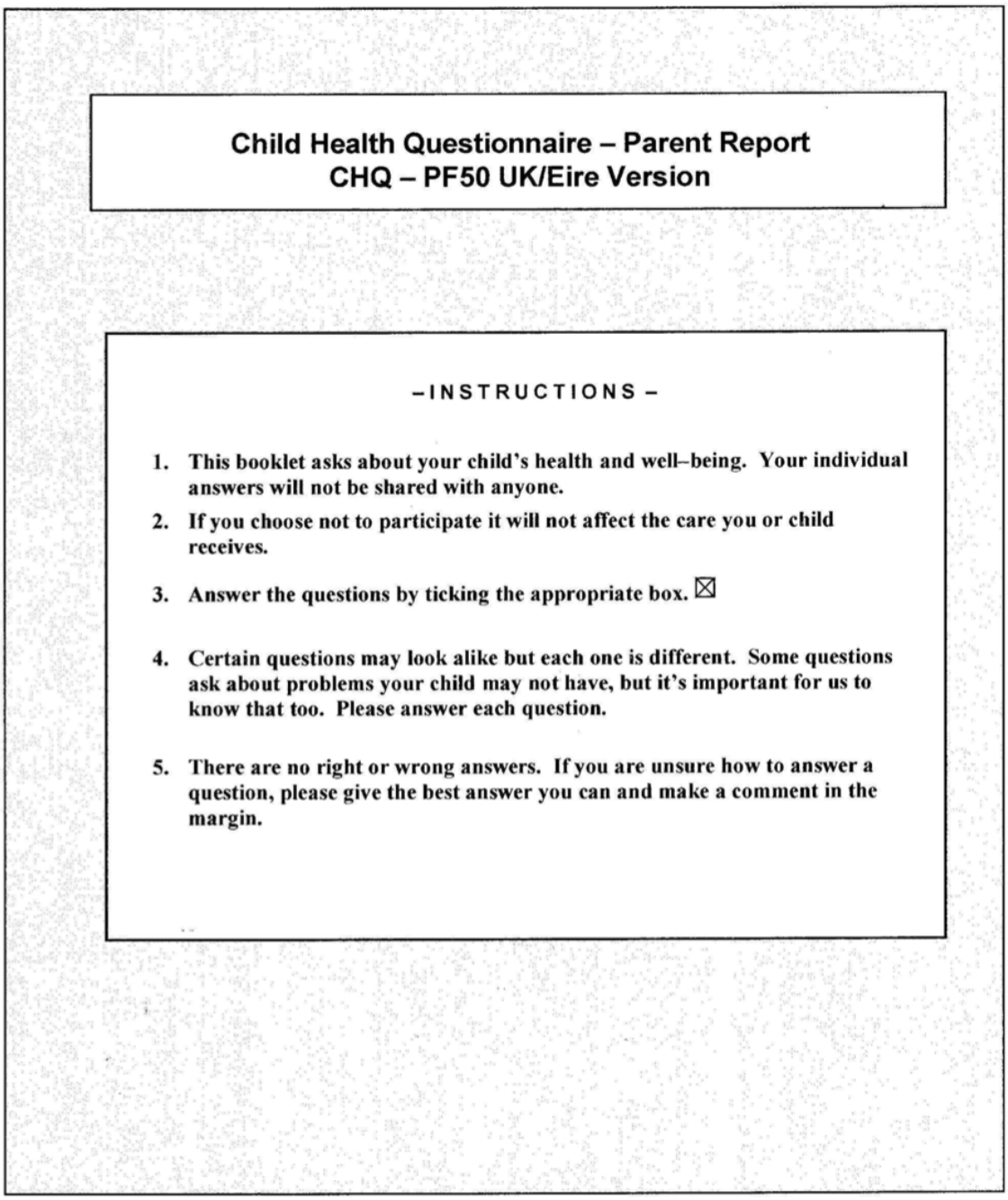
9.1. During the past 4 weeks, how often has your health or behaviour:

	Very often	Fairly often	Sometimes	Almost never	Never
a. limited the types of activities you could do as a family?	☐	☐	☐	☐	☐
b. interrupted various everyday family activities (eating meals, watching tv)?	☐	☐	☐	☐	☐
c. limited your ability as a family to "pick up and go" on a moment's notice?	☐	☐	☐	☐	☐
d. caused tension or conflict in your home?	☐	☐	☐	☐	☐
e. been a source of disagreements or arguments in your family?	☐	☐	☐	☐	☐
f. caused your family to cancel or change plans at the last minute?	☐	☐	☐	☐	☐

9.2. Sometimes families may have difficulty getting along with one another. They do not always agree and they may get angry. In general, how would you rate your family's ability to get along with one another?

☐ Excellent ☐ Very good ☐ Good ☐ Fair ☐ Poor

3.4.1.2 CHQ-PF50



Child Health Questionnaire – Parent Report
CHQ – PF50 UK/Eire Version

– INSTRUCTIONS –

1. **This booklet asks about your child's health and well–being. Your individual answers will not be shared with anyone.**
2. **If you choose not to participate it will not affect the care you or child receives.**
3. **Answer the questions by ticking the appropriate box. ☒**
4. **Certain questions may look alike but each one is different. Some questions ask about problems your child may not have, but it's important for us to know that too. Please answer each question.**
5. **There are no right or wrong answers. If you are unsure how to answer a question, please give the best answer you can and make a comment in the margin.**

1

SECTION #1: YOUR CHILD'S GLOBAL HEALTH

1.1. In general, would you say <u>your child's health</u> is:

☐ Excellent ☐ Very good ☐ Good ☐ Fair ☐ Poor

SECTION #2: YOUR CHILD'S PHYSICAL ACTIVITIES

The following questions ask about physical activities your child might do during a day.

2.1. During the <u>past 4 weeks</u>, has your child been limited in any of the following activities due to <u>health problems</u>?

	Yes, limited a lot	Yes, limited some	Yes, limited a little	No, not limited
a. Doing things that take **a lot** of energy, such as playing football or running?	☐	☐	☐	☐
b. Doing things that take **some** energy such as riding a bike?	☐	☐	☐	☐
c. Ability (physically) to get around the neighbourhood, playground, or school?	☐	☐	☐	☐
d. Walking 100 metres or climbing one flight of stairs?	☐	☐	☐	☐
e. Bending, lifting, or stooping?	☐	☐	☐	☐
f. Taking care of him/herself, that is, eating, dressing, bathing, or going to the toilet?	☐	☐	☐	☐

SECTION #3: YOUR CHILD'S EVERYDAY ACTIVITIES

3.1. During the <u>past 4 weeks</u>, has your child's school work or activities with friends been limited in any of the following ways due to EMOTIONAL difficulties or problems with his/her BEHAVIOUR?

	Yes, limited a lot	Yes, limited some	Yes, limited a little	No, not limited
a. limited in the KIND of school work or activities with friends he/she could do	☐	☐	☐	☐
b. limited in the AMOUNT of time he/she could spend on school work or activities with friends	☐	☐	☐	☐
c. limited in PERFORMING school work or activities with friends (it took extra effort)	☐	☐	☐	☐

3.2. During the <u>past 4 weeks</u>, has your child's school work or activities with friends been limited in any of the following ways due to problems with his/her PHYSICAL health?

	Yes, limited a lot	Yes, limited some	Yes, limited a little	No, not limited
a. limited in the KIND of school work or activities with friends he/she could do	☐	☐	☐	☐
b. limited in the AMOUNT of time he/she could spend on school work or activities with friends	☐	☐	☐	☐

SECTION #4: PAIN

4.1. During the past 4 weeks, how much bodily pain/discomfort has your child had?

☐ None ☐ Very mild ☐ Mild ☐ Moderate ☐ Severe ☐ Very severe

4.2 During the past 4 weeks, how often has your child had bodily pain/ discomfort?

☐ None of the time ☐ Once or twice ☐ A few times ☐ Fairly often ☐ Very often ☐ Every/almost every day

SECTION #5: BEHAVIOUR

Below is a list of items that describe children's behaviour or problems they sometimes have.

5.1. How often during the past 4 weeks did each of the following statements describe your child?

	Very often	Fairly often	Sometimes	Almost never	Never
a. argued a lot	☐	☐	☐	☐	☐
b. had difficulty concentrating or paying attention	☐	☐	☐	☐	☐
c. lied or cheated	☐	☐	☐	☐	☐
d. stole things inside or outside the home	☐	☐	☐	☐	☐
e. had tantrums or a hot temper	☐	☐	☐	☐	☐

5.2. Compared to other children your child's age, in general would you say his/her behaviour is:

☐ Excellent ☐ Very good ☐ Good ☐ Fair ☐ Poor

SECTION #6: WELL-BEING

The following phrases are about children's moods.

6.1. During the past 4 weeks, how much of the time do you think your child:

	All of the time	Most of the time	Some of the time	A little of the time	None of the time
a. felt like crying?	☐	☐	☐	☐	☐
b. felt lonely?	☐	☐	☐	☐	☐
c. acted nervous?	☐	☐	☐	☐	☐
d. acted bothered or upset?	☐	☐	☐	☐	☐
e. acted cheerful?	☐	☐	☐	☐	☐

SECTION #7: SELF–ESTEEM

The following ask about your child's satisfaction with self, school, and others. It may be helpful if you keep in mind how other children your child's age might feel about these areas.

7.1. During the past 4 weeks, how satisfied do you think your child has felt about:

	Very satisfied	Somewhat satisfied	Neither satisfied nor dissatisfied	Somewhat dissatisfied	Very dissatisfied
a. his/her school ability?	☐	☐	☐	☐	☐
b. his/her athletic ability?	☐	☐	☐	☐	☐
c. his/her friendships?	☐	☐	☐	☐	☐
d. his/her looks/appearance?	☐	☐	☐	☐	☐
e. his/her family relationships?	☐	☐	☐	☐	☐
f. his/her life overall?	☐	☐	☐	☐	☐

SECTION #8: YOUR CHILD'S HEALTH

The following statements are about health in general.

8.1. How true or false is each of these statements for your child?

	Definitely true	Mostly true	Don't know	Mostly false	Definitely false
a. My child seems to be less healthy than other children I know.	☐	☐	☐	☐	☐
b. My child has never been seriously ill.	☐	☐	☐	☐	☐
c. When there is something going around my child usually catches it.	☐	☐	☐	☐	☐
d. I expect my child will have a very healthy life.	☐	☐	☐	☐	☐
e. I worry more about my child's health than other people worry about their children's health.	☐	☐	☐	☐	☐

8.2. Compared to one year ago, how would you rate your child's health now:

☐ Much better now than 1 year ago	☐ Somewhat better now than 1 year ago	☐ About the same now as 1 year ago	☐ Somewhat worse now than 1 year ago	☐ Much worse now than 1 year ago

SECTION #9: YOU AND YOUR FAMILY

9.1. During the <u>past 4 weeks</u>, how MUCH emotional worry or concern did each of the following cause YOU?

	None at all	A little bit	Some	Quite a bit	A lot
a. Your child's physical health	☐	☐	☐	☐	☐
b. Your child's emotional well–being or behaviour	☐	☐	☐	☐	☐
c. Your child's attention or learning abilities	☐	☐	☐	☐	☐

9.2. During the <u>past 4 weeks</u>, were you LIMITED in the amount of time YOU had for your own needs because of:

	Yes, limited a lot	Yes, limited some	Yes, limited a little	No Not lir
a. Your child's physical health?	☐	☐	☐	☐
b. Your child's emotional well–being or behaviour?	☐	☐	☐	☐
c. Your child's attention or learning abilities?	☐	☐	☐	☐

9.3. During the past 4 weeks, <u>how often</u> has your child's <u>health or behaviour</u>:

	Very often	Fairly often	Sometimes	Almost never	Never
a. Limited the types of activities you could do as a family?	☐	☐	☐	☐	☐
b. Interrupted various everyday family activities (eating meals, watching TV)?	☐	☐	☐	☐	☐
c. Limited your ability as a family to "drop everything" on a moment's notice?	☐	☐	☐	☐	☐
d. Caused tension or conflict in your home?	☐	☐	☐	☐	☐
e. Been a source of disagreements or arguments in your family?	☐	☐	☐	☐	☐
f. Caused you to cancel or change plans (personal or work) at the last minute?	☐	☐	☐	☐	☐

9.4. Sometimes families may have difficulty getting along with one another. They do not always agree and they may get angry. In general, how would you rate your family's ability to get along with one another?

☐ Excellent ☐ Very good ☐ Good ☐ Fair ☐ Poor

3.4.2 Piers Harris 2

1. My classmates make fun of me.	yes	no
2. I am a happy person.	yes	no
3. It is hard for me to make friends.	yes	no
4. I am often sad.	yes	no
5. I am smart.	yes	no
6. I am shy.	yes	no
7. I get nervous when the teacher calls on me.	yes	no
8. My looks bother me.	yes	no
9. I am a leader in games and sports.	yes	no
10. I get worried when we have tests in school.	yes	no
11. I am unpopular.	yes	no
12. I am well behaved in school.	yes	no
13. It is usually my fault when something goes wrong.	yes	no
14. I cause trouble to my family.	yes	no
15. I am strong.	yes	no
16. I am an important member of my family.	yes	no
17. I give up easily.	yes	no
18. I am good in my schoolwork.	yes	no
19. I do many bad things.	yes	no
20. I behave badly at home.	yes	no
21. I am slow in finishing my schoolwork.	yes	no
22. I am an important member of my class.	yes	no
23. I am nervous.	yes	no
24. I can give a good report in front of the class.	yes	no
25. In school I am a dreamer.	yes	no
26. My friends like my ideas.	yes	no
27. I often get into trouble.	yes	no
28. I am lucky.	yes	no
29. I worry a lot.	yes	no
30. My parents expect too much of me.	yes	no
31. I like being the way I am.	yes	no

The Way I Feel About Myself

PIERS-HARRIS 2

AutoScore™ Form

by Ellen V. Piers, Ph.D., Dale B. Harris, Ph.D., & David S. Herzberg, Ph.D.

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Los Angeles, CA 90025-1251
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Client's Name (or ID #): _____

Today's Date: _____ Age: _____

Gender: *(circle one)* Female Male Grade: _____

School: _____

Teacher's Name (optional): _____

Race/Ethnicity: ☐ Asian ☐ Hispanic ☐ White
☐ Black ☐ Native American ☐ Other

Directions

Here are some sentences that tell how some people feel about themselves. Read each sentence and decide whether it tells the way you feel about yourself. If it is *true* or *mostly true* for you, circle the word *yes* next to the statement. If it is *false* or *mostly false* for you, circle the word *no*. Answer every question, even if some are hard to decide. Do not circle both *yes* and *no* for the same sentence. If you want to change your answer, cross it out with an X and circle your new answer.

Remember that there are no right or wrong answers. Only you can tell us how you feel about yourself, so we hope you will mark each sentence the way you really feel inside.

W-388A

32. I feel left out of things. ..... yes no

33. I have nice hair. ..... yes no

34. I often volunteer in school. ..... yes no

35. I wish I were different. ..... yes no

36. I hate school. ..... yes no

37. I am among the last to be chosen for games and sports. ..... yes no

38. I am often mean to other people. ..... yes no

39. My classmates in school think I have good ideas. ..... yes no

40. I am unhappy. ..... yes no

41. I have many friends. ..... yes no

42. I am cheerful. ..... yes no

43. I am dumb about most things. ..... yes no

44. I am good-looking. ..... yes no

45. I get into a lot of fights. ..... yes no

46. I am popular with boys. ..... yes no

47. People pick on me. ..... yes no

48. My family is disappointed in me. ..... yes no

49. I have a pleasant face. ..... yes no

50. When I grow up, I will be an important person. ..... yes no

51. In games and sports, I watch instead of play. ..... yes no

52. I forget what I learn. ..... yes no

53. I am easy to get along with. ..... yes no

54. I am popular with girls. ..... yes no

55. I am a good reader. ..... yes no

56. I am often afraid. ..... yes no

57. I am different from other people. ..... yes no

58. I think bad thoughts. ..... yes no

59. I cry easily. ..... yes no

60. I am a good person. ..... yes no

3.4.3 The Pediatric Quality Of Life Inventory (PedsQL)

Child 5-7 year old

ID# _____

Date:_____

PedsQL™

Paediatric Quality of Life Inventory

Version 4.0 – UK English

YOUNG CHILD REPORT (ages **5-7**)

Instructions for interviewer:

I am going to ask you some questions about things that might be a problem for some children. I want to know how much of a problem any of these things might be for you.

Show the child the template and point to the responses as you read.

If it is <u>not at all</u> a problem for you, point to the smiling face.

If it is <u>sometimes</u> a problem for you, point to the middle face.

If it is a problem for you <u>a lot</u>, point to the frowning face.

I will read each question. Point to the pictures to show me how much of a problem it is for you. Let's try a practice one first.

	Not at all	Sometimes	A lot
Is it hard for you to click your fingers?	☺	😐	☹

Ask the child to demonstrate clicking his or her fingers to determine whether or not the question was answered correctly. Repeat the question if the child demonstrates a response that is different from his or her action.

Think about how you have been doing for the last few weeks. Please listen carefully to each sentence and tell me how much of a problem this is for you.

After reading the item, gesture to the template. If the child hesitates or does not seem to understand how to answer, read the response options while pointing at the faces.

PHYSICAL FUNCTIONING ***(problems with…)***	**Not at all**	**Some-times**	**A lot**
1. Is it hard for you to walk?	0	2	4
2. Is it hard for you to run?	0	2	4
3. Is it hard for you to play sports or exercise?	0	2	4
4. Is it hard for you to lift big things?	0	2	4
5. Is it hard for you to have a bath or shower?	0	2	4
6. Is it hard for you to help in the home (like picking up your toys)?	0	2	4
7. Do you have aches and pains? (***Where?_____***)	0	2	4
8. Do you ever feel too tired to play?	0	2	4

Remember, tell me how much of a problem this has been for you for the last few weeks.

EMOTIONAL FUNCTIONING ***(problems with…)***	**Not at all**	**Some-times**	**A lot**
1. Do you feel scared?	0	2	4
2. Do you feel sad?	0	2	4
3. Do you feel angry?	0	2	4
4. Do you have trouble sleeping?	0	2	4
5. Do you worry about what will happen to you?	0	2	4

SOCIAL FUNCTIONING ***(problems with…)***	**Not at all**	**Some-times**	**A lot**
1. Do you have trouble getting on with other children?	0	2	4
2. Do other children say they do not want to play with you?	0	2	4
3. Do other children tease you?	0	2	4
4. Can other children do things you cannot do?	0	2	4
5. Is it hard for you to keep up when you play with other children?	0	2	4

SCHOOL FUNCTIONING ***(problems with…)***	**Not at all**	**Some-times**	**A lot**
1. Is it hard for you to pay attention in school?	0	2	4
2. Do you forget things?	0	2	4
3. Do you have trouble keeping up with schoolwork?	0	2	4
4. Do you miss school because of not feeling well?	0	2	4
5. Do you miss school to go to the doctor or hospital?	0	2	4

How much of a problem is this for you?

Not at all

Sometimes

A lot

Child 8-12 year old

ID# _____

Date:_____

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CHILD REPORT (ages **8-12**)

DIRECTIONS

On the following page is a list of things that might be a problem for you. Please tell us **how much of a problem** each one has been for you during the **PAST MONTH** by circling:

0 if it is **never** a problem
1 if it is **almost never** a problem
2 if it is **sometimes** a problem
3 if it is **often** a problem
4 if it is **almost always** a problem

There are no right or wrong answers.
If you do not understand a question, please ask for help.

*In the **PAST MONTH**, how much of a **problem** has this been for you …*

ABOUT MY HEALTH AND ACTIVITIES ***(problems with…)***	**Never**	**Almost Never**	**Some-times**	**Often**	**Almost Always**
1. It is hard for me to walk more than a couple of streets (about 100 metres)	0	1	2	3	4
2. It is hard for me to run	0	1	2	3	4
3. It is hard for me to do sports activities or exercise	0	1	2	3	4
4. It is hard for me to lift heavy things	0	1	2	3	4
5. It is hard for me to have a bath or shower by myself	0	1	2	3	4
6. It is hard for me to do chores around the house	0	1	2	3	4
7. I have aches and pains	0	1	2	3	4
8. I feel tired	0	1	2	3	4

ABOUT MY FEELINGS ***(problems with…)***	**Never**	**Almost Never**	**Some-times**	**Often**	**Almost Always**
1. I feel afraid or scared	0	1	2	3	4
2. I feel sad	0	1	2	3	4
3. I feel angry	0	1	2	3	4
4. I have trouble sleeping	0	1	2	3	4
5. I worry about what will happen to me	0	1	2	3	4

HOW I GET ON WITH OTHERS ***(problems with…)***	**Never**	**Almost Never**	**Some-times**	**Often**	**Almost Always**
1. I have trouble getting on with other children	0	1	2	3	4
2. Other children do not want to be my friend	0	1	2	3	4
3. Other children tease me	0	1	2	3	4
4. I cannot do things that other children my age can do	0	1	2	3	4
5. It is hard to keep up when I play with other children	0	1	2	3	4

ABOUT SCHOOL ***(problems with…)***	**Never**	**Almost Never**	**Some-times**	**Often**	**Almost Always**
1. It is hard to pay attention in class	0	1	2	3	4
2. I forget things	0	1	2	3	4
3. I have trouble keeping up with my schoolwork	0	1	2	3	4
4. I miss school because of not feeling well	0	1	2	3	4
5. I miss school to go to the doctor or hospital	0	1	2	3	4

Child 13-18 year old

ID# _____

Date:_____

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Paediatric Quality of Life Inventory

Version 4.0 – UK English

TEENAGER REPORT (ages **13-18**)

DIRECTIONS

On the following page is a list of things that might be a problem for you. Please tell us **how much of a problem** each one has been for you during the **PAST MONTH** by circling:

0 if it is **never** a problem
1 if it is **almost never** a problem
2 if it is **sometimes** a problem
3 if it is **often** a problem
4 if it is **almost always** a problem

There are no right or wrong answers.
If you do not understand a question, please ask for help.

*In the **PAST MONTH**, how much of a **problem** has this been for you …*

ABOUT MY HEALTH AND ACTIVITIES ***(problems with…)***	**Never**	**Almost Never**	**Some-times**	**Often**	**Almost Always**
1. It is hard for me to walk more than a couple of streets (about 100 metres)	0	1	2	3	4
2. It is hard for me to run	0	1	2	3	4
3. It is hard for me to do sports activities or exercise	0	1	2	3	4
4. It is hard for me to lift heavy things	0	1	2	3	4
5. It is hard for me to have a bath or shower by myself	0	1	2	3	4
6. It is hard for me to do chores around the house	0	1	2	3	4
7. I have aches and pains	0	1	2	3	4
8. I feel tired	0	1	2	3	4

ABOUT MY FEELINGS ***(problems with…)***	**Never**	**Almost Never**	**Some-times**	**Often**	**Almost Always**
1. I feel afraid or scared	0	1	2	3	4
2. I feel sad	0	1	2	3	4
3. I feel angry	0	1	2	3	4
4. I have trouble sleeping	0	1	2	3	4
5. I worry about what will happen to me	0	1	2	3	4

HOW I GET ON WITH OTHERS ***(problems with…)***	**Never**	**Almost Never**	**Some-times**	**Often**	**Almost Always**
1. I have trouble getting on with other teenagers	0	1	2	3	4
2. Other teenagers do not want to be my friend	0	1	2	3	4
3. Other teenagers tease me	0	1	2	3	4
4. I cannot do things that other teenagers my age can do	0	1	2	3	4
5. It is hard to keep up with other teenagers my age	0	1	2	3	4

ABOUT SCHOOL / COLLEGE ***(problems with…)***	**Never**	**Almost Never**	**Some-times**	**Often**	**Almost Always**
1. It is hard to pay attention in class	0	1	2	3	4
2. I forget things	0	1	2	3	4
3. I have trouble keeping up with my school / college work	0	1	2	3	4
4. I miss school / college because of not feeling well	0	1	2	3	4
5. I miss school / college to go to the doctor or hospital	0	1	2	3	4

Parent of child 5-7 year old

ID#
_____

Date:_____
_

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Version 4.0

PARENT REPORT for **YOUNG CHILDREN** (ages **5-7**)

DIRECTIONS

On the following page is a list of things that might be a problem for **your child**. Please tell us **how much of a problem** each one has been for **your child** during the **past ONE month** by circling:

0 if it is **never** a problem
1 if it is **almost never** a problem
2 if it is **sometimes** a problem
3 if it is **often** a problem
4 if it is **almost always** a problem

There are no right or wrong answers.
If you do not understand a question, please ask for help.

*In the past **ONE month,** how much of a **problem** has your child had with …*

PHYSICAL FUNCTIONING (PROBLEMS WITH…)	Never	Almost Never	Some-times	Often	Almost Always
1. Walking down the road a little bit	0	1	2	3	4
2. Running	0	1	2	3	4
3. Participating in sports or running games	0	1	2	3	4
4. Lifting heavy things	0	1	2	3	4
5. Having a bath or shower by him or herself	0	1	2	3	4
6. Helping to pick up his or her toys	0	1	2	3	4
7. Having hurts or aches	0	1	2	3	4
8. Feeling very tired	0	1	2	3	4

EMOTIONAL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Feeling afraid or scared	0	1	2	3	4
2. Feeling sad or unhappy	0	1	2	3	4
3. Feeling angry or cross	0	1	2	3	4
4. Trouble sleeping at night	0	1	2	3	4
5. Worrying about what will happen to him or her	0	1	2	3	4

SOCIAL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Getting on with other children	0	1	2	3	4
2. Other kids not wanting to be his or her friend	0	1	2	3	4
3. Getting bullied by other children	0	1	2	3	4
4. Not able to do things that other children his or her age can do	0	1	2	3	4
5. Keeping up when playing with other children	0	1	2	3	4

SCHOOL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Paying attention in class	0	1	2	3	4
2. Forgetting things	0	1	2	3	4
3. Keeping up with school activities	0	1	2	3	4
4. Having days off school because of not feeling well	0	1	2	3	4
5. Having days off school to go to the doctor or hospital	0	1	2	3	4

Parent of child 8-12 year old

ID#_____

Date:_____

PedsQL™
Paediatric Quality of Life Inventory

Version 4.0 - UK English

PARENT REPORT for **CHILDREN** (ages **8-12**)

DIRECTIONS

On the following page is a list of things that might be a problem for **your child**. Please tell us **how much of a problem** each one has been for **your child** during the **past ONE month** by circling:

0 if it is **never** a problem
1 if it is **almost never** a problem
2 if it is **sometimes** a problem
3 if it is **often** a problem
4 if it is **almost always** a problem

There are no right or wrong answers.
If you do not understand a question, please ask for help.

*In the past **ONE month,** how much of a **problem** has your child had with*

PHYSICAL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Walking 100 metres	0	1	2	3	4
2. Running	0	1	2	3	4
3. Participating in sports activities or exercise	0	1	2	3	4
4. Lifting something heavy	0	1	2	3	4
5. Taking a bath or shower by him or herself	0	1	2	3	4
6. Doing chores around the house	0	1	2	3	4
7. Having aches or pains	0	1	2	3	4
8. Low energy levels	0	1	2	3	4

EMOTIONAL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Feeling afraid or scared	0	1	2	3	4
2. Feeling sad	0	1	2	3	4
3. Feeling angry	0	1	2	3	4
4. Trouble sleeping	0	1	2	3	4
5. Worrying about what will happen to him or her	0	1	2	3	4

SOCIAL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Getting on with other children	0	1	2	3	4
2. Other children not wanting to be his or her friend	0	1	2	3	4
3. Getting teased by other children	0	1	2	3	4
4. Not being able to do things that other children his or her age can do	0	1	2	3	4
5. Keeping up when playing with other children	0	1	2	3	4

SCHOOL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Paying attention in class	0	1	2	3	4
2. Forgetting things	0	1	2	3	4
3. Keeping up with schoolwork	0	1	2	3	4
4. Missing school because of not feeling well	0	1	2	3	4
5. Missing school to go to the doctor or hospital	0	1	2	3	4

Parent of child 13-18 year old

ID#_____

Date:_____

PedsQL™

Paediatric Quality of Life Inventory

Version 4.0 English (United Kingdom)

PARENT REPORT for TEENAGERS (ages 13-18)

INSTRUCTIONS

On the following page is a list of things that might be a problem for **your teenager**.
Please tell us **how much of a problem** each one has been for **your teenager** during the **past ONE month** by circling:

0 if it is **never** a problem
1 if it is **almost never** a problem
2 if it is **sometimes** a problem
3 if it is **often** a problem
4 if it is **almost always** a problem

There are no right or wrong answers.
If you do not understand a question, please ask for help.

PedsQL PA - United Kingdom/English - Version of 14 Mar 11 - Mapi Research Institute.
ID6014 / PedsQL-4.0-Core-PA_AU4.0_eng-GB.doc

*In the past **ONE month**, how much of a **problem** has your teenager had with …*

PHYSICAL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Walking 100 metres	0	1	2	3	4
2. Running	0	1	2	3	4
3. Participating in sports activities or exercise	0	1	2	3	4
4. Lifting something heavy	0	1	2	3	4
5. Taking a bath or shower by him or herself	0	1	2	3	4
6. Doing chores around the house	0	1	2	3	4
7. Having aches or pains	0	1	2	3	4
8. Feeling tired	0	1	2	3	4

EMOTIONAL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Feeling afraid or scared	0	1	2	3	4
2. Feeling sad	0	1	2	3	4
3. Feeling angry	0	1	2	3	4
4. Trouble sleeping	0	1	2	3	4
5. Worrying about what will happen to him or her	0	1	2	3	4

SOCIAL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Getting on with other teenagers	0	1	2	3	4
2. Other teenagers not wanting to be his or her friend	0	1	2	3	4
3. Getting teased by other teenagers	0	1	2	3	4
4. Not being able to do things that other teenagers his or her age can do	0	1	2	3	4
5. Keeping up with other teenagers	0	1	2	3	4

SCHOOL FUNCTIONING *(problems with…)*	Never	Almost Never	Some-times	Often	Almost Always
1. Paying attention in class	0	1	2	3	4
2. Forgetting things	0	1	2	3	4
3. Keeping up with schoolwork	0	1	2	3	4
4. Missing school because of not feeling well	0	1	2	3	4
5. Missing school to go to the doctor or hospital	0	1	2	3	4

3.4.4 The Pediatric Inventory for Parents (Pip)

Patient ID#:_____ Patient Initials:_____ Date Form Completed:_____/_____/_____

PEDIATRIC INVENTORY FOR PARENTS

Below is a list of difficult events which parents of children who have (or have had) a serious illness sometimes face. Please read each event carefully, and circle HOW OFTEN the event has occurred for you in the past 7 days, using the 5 point scale below. Afterwards, please rate how DIFFICULT it was/or generally is for you, also using the 5 point scale. Please complete both columns for each item.

EVENT	HOW OFTEN? 1=Never, 2=Rarely, 3=Sometimes, 4=Often, 5=Very often	HOW DIFFICULT? 1=Not at all, 2=A little, 3=Somewhat, 4=Very much, 5=Extremely
1. Difficulty sleeping	1 2 3 4 5	1 2 3 4 5
2. Arguing with family member(s)	1 2 3 4 5	1 2 3 4 5
3. Bringing my child to the clinic or hospital	1 2 3 4 5	1 2 3 4 5
4. Learning upsetting news	1 2 3 4 5	1 2 3 4 5
5. Being unable to go to work/job	1 2 3 4 5	1 2 3 4 5
6. Seeing my child's mood change quickly	1 2 3 4 5	1 2 3 4 5
7. Speaking with doctor	1 2 3 4 5	1 2 3 4 5
8. Watching my child have trouble eating	1 2 3 4 5	1 2 3 4 5
9. Waiting for my child's test results	1 2 3 4 5	1 2 3 4 5
10. Having money/financial troubles	1 2 3 4 5	1 2 3 4 5
11. Trying not to think about my family's difficulties	1 2 3 4 5	1 2 3 4 5
12. Feeling confused about medical information	1 2 3 4 5	1 2 3 4 5
13. Being with my child during medical procedures	1 2 3 4 5	1 2 3 4 5
14. Knowing my child is hurting or in pain	1 2 3 4 5	1 2 3 4 5
15. Trying to attend to the needs of other family members	1 2 3 4 5	1 2 3 4 5
16. Seeing my child sad or scared	1 2 3 4 5	1 2 3 4 5
17. Talking with the nurse	1 2 3 4 5	1 2 3 4 5
18. Making decisions about medical care or medicines	1 2 3 4 5	1 2 3 4 5
19. Thinking about my child being isolated from others	1 2 3 4 5	1 2 3 4 5
20. Being far away from family and/or friends	1 2 3 4 5	1 2 3 4 5
21. Feeling numb inside	1 2 3 4 5	1 2 3 4 5
22. Disagreeing with a member of the health care team	1 2 3 4 5	1 2 3 4 5

Patient ID:_____ Patient Initials#:_____

EVENT	HOW OFTEN? 1=Never, 2=Rarely, 3=Sometimes, 4=Often, 5=Very often	HOW DIFFICULT? 1=Not at all, 2=A little, 3=Somewhat, 4=Very much, 5=Extremely
23. Helping my child with his/her hygiene needs	1 2 3 4 5	1 2 3 4 5
24. Worrying about the long term impact of the illness	1 2 3 4 5	1 2 3 4 5
25. Having little time to take care of my own needs	1 2 3 4 5	1 2 3 4 5
26. Feeling helpless over my child's condition	1 2 3 4 5	1 2 3 4 5
27. Feeling misunderstood by family/friends as to the severity of my child's illness	1 2 3 4 5	1 2 3 4 5
28. Handling changes in my child's daily medical routines	1 2 3 4 5	1 2 3 4 5
29. Feeling uncertain about the future	1 2 3 4 5	1 2 3 4 5
30. Being in the hospital over weekends/holidays	1 2 3 4 5	1 2 3 4 5
31. Thinking about other children who have been seriously ill	1 2 3 4 5	1 2 3 4 5
32. Speaking with child about his/her illness	1 2 3 4 5	1 2 3 4 5
33. Helping my child with medical procedures (e.g. giving shots, swallowing medicine, changing dressing)	1 2 3 4 5	1 2 3 4 5
34. Having my heart beat fast, sweating, or feeling tingly	1 2 3 4 5	1 2 3 4 5
35. Feeling uncertain about disciplining my child	1 2 3 4 5	1 2 3 4 5
36. Feeling scared that my child could get very sick or die	1 2 3 4 5	1 2 3 4 5
37. Speaking with family members about my child's illness	1 2 3 4 5	1 2 3 4 5
38. Watching my child during procedures	1 2 3 4 5	1 2 3 4 5
39. Missing important events in the lives of other family members.	1 2 3 4 5	1 2 3 4 5
40. Worrying about how friends and relatives interact with my child	1 2 3 4 5	1 2 3 4 5
41. Noticing a change in my relationship with my partner	1 2 3 4 5	1 2 3 4 5
42. Spending a great deal of time in unfamiliar settings	1 2 3 4 5	1 2 3 4 5

3.4.5 The Hospital Anxiety and Depression scale (HAD)

We are aware that emotions play an important part in most illnesses. If we know about these feelings we will be able to help you more. This questionnaire is designed to help us know how you have been feeling for the last 2 weeks. **Please answer all the questions**. If you are unsure about which response to give to a question, **please choose the ONE** that appears most appropriate. This can often be your first response.
TICK ONLY ONE BOX PER QUESTION
Don't take too long over your replies: your immediate reaction to each item will probably be more accurate than a long thought out response.

		Most of the time	A lot of the time	Time to time, occasionally	Not at all
1	I feel tense or wound up	1	2	3	4

		Definitely as much	Not quite so much	Only a little	Not at all
2	I enjoy the things I used to enjoy	1	2	3	4

		Very definitely & quite badly	Yes, but not too badly	Definitely not so much now	Not at all
3	I get a sort of frightened feeling as if something awful is about to happen	1	2	3	4

		As much as I always could	Not quite so much	Definitely not so much	Not at all
4	I can laugh and see the funny side of things	1	2	3	4

		A great deal of the time	A lot of the time	From time to time not too often	Only occasionally
4	Worrying thoughts go through my mind	1	2	3	4

		Not at all	Not often	Sometimes	Most of the time
6	I feel cheerful	1	2	3	4

		Definitely	Usually	Not often	Not at all
7	I can sit at ease and feel relaxed	1	2	3	4

		Nearly all the time	Very often	Sometimes	Not at all
8	I feel as if I am slowed down	1	2	3	4

		Most of the time	A lot of the time	Time to time, occasionally	Not at all
9	I get a sort of frightened feeling like 'butterflies' in the stomach	1	2	3	4

		Definitely	I don't care so much as I should	I may not take quite as much care	I take just as much care as ever
10	I have lost interest in my appearance	1	2	3	4

		Very much indeed	Quite a lot	Not very often	Not at all
11	I feel restless as if I have to be on the move	1	2	3	4

		As much as I ever did	Rather less than I used to	Definitely less that I used to	Hardly at all
12	I look forward with enjoyment to things	1	2	3	4

		Very often indeed	Quite often	Not very often	Not at all
13	I get sudden feelings of panic	1	2	3	4

		Often	Sometimes	Not often	Very seldom
14	I can enjoy a good book or radio or TV programme	1	2	3	4

3.4.6 The Physical Activity questionnaire (PAQ)

Physical Activity Questionnaire (Elementary School)

Name:_____ Age:_____

Sex: M_____ F_____ Grade:_____

Teacher:_____

We are trying to find out about your level of physical activity from ***the last 7 days*** (in the last week). This includes sports or dance that make you sweat or make your legs feel tired, or games that make you breathe hard, like tag, skipping, running, climbing, and others.

Remember:

1. There are no right and wrong answers — this is not a test.
2. Please answer all the questions as honestly and accurately as you can — this is very important.

---

1. Physical activity in your spare time: Have you done any of the following activities in the past 7 days (last week)? If yes, how many times? (Mark only one circle per row.)

	No	1-2	3-4	5-6	7 times or more
Skipping	○	○	○	○	○
Rowing/canoeing	○	○	○	○	○
In-line skating	○	○	○	○	○
Tag	○	○	○	○	○
Walking for exercise	○	○	○	○	○
Bicycling	○	○	○	○	○
Jogging or running	○	○	○	○	○
Aerobics	○	○	○	○	○
Swimming	○	○	○	○	○
Baseball, softball	○	○	○	○	○
Dance	○	○	○	○	○
Football	○	○	○	○	○
Badminton	○	○	○	○	○
Skateboarding	○	○	○	○	○
Soccer	○	○	○	○	○
Street hockey	○	○	○	○	○
Volleyball	○	○	○	○	○
Floor hockey	○	○	○	○	○
Basketball	○	○	○	○	○
Ice skating	○	○	○	○	○
Cross-country skiing	○	○	○	○	○
Ice hockey/ringette	○	○	○	○	○
Other:					
_____	○	○	○	○	○
_____	○	○	○	○	○

8

2. In the last 7 days, during your physical education (PE) classes, how often were you very active (playing hard, running, jumping, throwing)? (Check one only.)
I don't do PE
Hardly ever
Sometimes
Quite often
Always

3. In the last 7 days, what did you normally do *at lunch* (besides eating lunch)? (Check one only.)
Sat down (talking, reading, doing schoolwork)
Stood around or walked around
Ran or played a little bit
Ran around and played quite a bit
Ran and played hard most of the time

4. In the last 7 days, on how many days *right after school*, did you do sport, dance, or play games in which you were very active? (Check one only.)
None
1 time last week
2 or 3 times last week
4 times last week
5 times last week

5. In the last 7 days, on how many *evenings* did you do sports, dance, or play games in which you were very active? (Check one only.)
None
1 time last week
2 or 3 times last week
4 or 5 last week
6 or 7 times last week.

6. *On the last weekend*, how many times did you do sports, dance, or play games in which you were very active? (Check one only.)
None
1 time
2 — 3 times
4 — 5 times
6 or more times

7. Which *one* of the following describes you best for the last 7 days? Read *all five* statements before deciding on the *one* answer that describes you.

A. All or most of my free time was spent doing things that involve little physical effort

B. I sometimes (1 — 2 times last week) did physical things in my free time

(e.g. played sports, went running, swimming, bike riding, did aerobics)

C. I often (3 — 4 times last week) did physical things in my free time

D. I quite often (5 — 6 times last week) did physical things in my free time

E. I very often (7 or more times last week) did physical things in my free time

8. Mark how often you did physical activity (like playing sports, games, doing dance, or any other physical activity) for each day last week.
None, very little, a bit, medium, often, very often
Monday
Tuesday
Wednesday
Thursday
Friday
Saturday
Sunday

9. Were you sick last week, or did anything prevent you from doing your normal physical activities? (Check one.)
Yes
No
If Yes, what prevented you? _____

Appendix for chapter 4

Table 4.1 NEM scale

Rating	Motor	Sensory	Bladder	Bowel
1	Wheelchair. *Major deficit•*	Skin ulceration or amputation	Day and night incontinence. Bladder augmentation/ mitrofanoff *Incontinence•*	**Incontinence / ostomy**
2	Major orthosis	Pain	Nocturnal incontinence. *Retention•*	**Painful constipation**
3	Distal orthosis. *Club foot, atrophy, distal deficit•*	Painless sensory deficit	CIC	**Normal**
4	Fatigue on walking	Normal	Dysuria, infections, stress incontinence	
5	Normal		Normal	

Table 4.2 Comparison of average PPQ ratings for females and males

Comparison of averaged PPQ-VAS ratings for female and males						
		Female (n=36)	**Male (n=18)**	**U**	**z**	**p**
Report	**Child** Mean IQR	 1 2.50	 **0.5** **2.50**	**254.5**	**-0.514**	**0.61**
	Parent Mean IQR	 **0.5** **4.50**	 **0** **3.00**	**297.5**	**-1.354**	**0.18**

Table 4.3 Comparison of averaged PPQ ratings for LSL type.

Comparison of averaged PPQ ratings (0 -10) for LSL type						
		Caudal (n=19)	**Dorsal** (n=15)	**Transitional** (n=20)	**x2**	**p**
Report	**Child** Mean IQR	 **0.50** 2.00	 **0.00** 3.00	 **2.00** 4.38	**2.78**	**0.25**
	Parent Mean IQR	 **0.00** 1.50	 **0.00** 4.50	 **3.0** 4.38	**3.46**	**0.18**

Table 4.4 Averaged PPQ-VAS ratings for patients without and with a syrinx

Averaged PPQ-VAS ratings for patients without and with a Syrinx						
		Median				
		No syrinx (n=38)	**Syrinx (n=16)**	**U**	**z**	**p**
Report	Child Mean IQR	 **1.5** **3.25**	 **0.00** **1.75**	**228**	**-1.52**	**0.13**
	Parent Mean IQR	 **1.0** **4.13**	 **0.00** **0.88**	**213**	**-1.83**	**0.07**

Appendix for chapter 5

Table 5.1 Tests of normality PedsQL parent and child

						Kolmogorov-Smirnov		
		n	Median	IQR	Skewness	Statistic	d.f.	p
Self (n=54)	Total	54	83.70	25.83	-1.027	0.137	54	0.013
	Physical	54	87.50	25.78	-1.235	0.201	54	<0.001
	Psychosocial	54	83.33	28.33	-0.744	0.144	54	0.007
	Emotional	54	80.00	40.00	-0.460	0.162	54	0.001
	Social	54	92.50	30.00	-1.379	0.226	54	<0.001
	School	54	87.50	35.00	-0.945	0.185	54	<0.001
Proxy (n=54)	Total	54	75.54	25.65	-0.871	0.099	54	0.200
	Physical	54	84.38	38.28	-0.994	0.171	54	<0.001
	Psychosocial	54	77.50	32.08	-0.724	0.109	54	0.156
	Emotional	54	75.00	32.50	-0.605	0.129	54	0.026
	Social	54	80.00	36.25	-0.880	0.150	54	0.004
	School	54	77.50	35.00	-0.919	0.166	54	0.001

Table 5.2 Correlations between PedsQL for self and parent

The diagonal includes coefficients for the correlation between self and proxy report. (Coefficients above the diagonal are self-report; coefficients below the diagonal are proxy report). Table 6.3 shows that all correlations were statistically significant, typically at the p<0.001 level.

Correlations between PedsQL domains for self and report (n=54).

	Total		Physical		Psychosocial		Emotional		Social		School	
	r_s	p	r_s	p	r_s	p	r_s	p	r_s	p	r_s	p
Total	0.726**	<0.001	0.792**	<0.001	0.951**	<0.001	0.761**	<0.001	0.720**	<0.001	0.727**	<0.001
Physical	0.825**	<0.001	0.743**	<0.001	0.589**	<0.001	0.398**	0.003	0.399**	0.003	0.518**	<0.001
Psychosocial	0.931**	<0.001	0.603**	<0.001	0.691**	<0.001	0.828**	<0.001	0.805**	<0.001	0.719**	<0.001
Emotional	0.783**	<0.001	0.549**	<0.01	0.814**	<0.001	0.627**	<0.001	0.591**	<0.001	0.333*	0.014
Social	0.809	<0.001	0.524**	<0.001	0.861**	<0.001	0.581**	<0.001	0.539**	<0.001	0.425**	0.001
School	0.703**	<0.001	0.409**	<0.002	0.775**	<0.001	0.436	<0.001	0.562**	<0.001	0.498**	<0.001

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.3 Comparison of LSL and healthy normative PedsQL data (Upton et al, 2005)

	LSL (n=54)		Normative (n=1399)		1-sample t-test		
	Mean	s.d.	Mean	s.d.	t	d.f.	p
Self-report							
Total	79.03	17.58	83.89	11.84	-2.03	53	0.047*
Physical health	78.30	22.04	88.51	11.62	-3.40	53	0.001**
Psychosocial health	79.41	17.45	81.84	13.21	-1.02	53	0.312
Emotional	75.37	22.23	78.49	17.94	-1.03	53	0.307
Social	83.98	20.64	87.65	16.46	-1.31	53	0.197
School	78.89	23.06	78.87	15.89	0.006	53	0.995
Proxy-report	(n=54)		(n=970)				
Total	74.30	19.94	84.61	11.19	-3.80	53	<0.001**
Physical health	74.94	26.39	89.06	12.27	-3.93	53	<0.001**
Psychosocial health	73.96	19.64	82.21	12.67	-3.09	53	0.003**
Emotional	71.67	24.07	78.28	15.54	-2.02	53	0.049*
Social	74.35	24.78	86.82	15.42	-3.70	53	0.001**
School	75.87	21.96	81.52	16.09	-1.90	53	0.064

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.4 PedsQL and child gender

Comparison of PedsQL scores by gender

	Female			Male			Mann-Whitney	
Self	n	Median	IQR	n	Median	IQR	U	p
Total	36	85.87	22.28	18	78.80	30.16	256.0	0.21
Physical	36	87.50	25.00	18	87.50	31.25	312.0	0.83
Psychosocial	36	87.50	27.50	18	79.17	33.75	234.5	0.10
Emotional	36	82.50	38.75	18	62.50	37.50	204.5*	0.026
Social	36	100.00	27.50	18	87.50	45.00	236.0	0.09
School	36	87.50	35.00	18	85.00	33.75	318.5	0.92
Proxy	N	Median	IQR	n	Median	IQR	U	p
Total	36	77.72	25.87	18	75.00	23.64	297.5	0.63
Physical	36	79.69	47.66	18	84.38	32.81	298.0	0.63
Psychosocial	36	80.00	29.17	18	73.33	30.42	269.0	0.31
Emotional	36	75.00	33.75	18	72.50	52.50	267.5	0.30
Social	36	82.50	37.50	18	72.50	36.25	263.0	0.26
School	36	75.00	33.75	18	85.00	37.50	316.5	0.89

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.5 Correlations between PedsQL self-scores and LSL groups

Statistical analysis of PedsQL self-scores by 3 LSL groups

	Kruskal-Wallis df=2, n=54		Mann-Whitney Caudal-Dorsal		Mann-Whitney Caudal-Transitional		Mann-Whitney Dorsal-Transitional	
	χ^2	P	U	p	U	p	U	p
Self-report								
Total	0.90	0.64	-	-	-	-	-	-
Physical	0.73	0.69	-	-	-	-	-	-
Psychosocial	1.37	0.51	-	-	-	-	-	-
Emotional	0.40	0.82	-	-	-	-	-	-
Social	2.22	0.33	-	-	-	-	-	-
School	0.40	0.82	-	-	-	-	-	-

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.6 Description, tests of normality, and one sample tests of CHQ-PF50 data

Description, tests of normality, and one sample tests of CHQ-PF50 data

					Kolmogorov-Smirnov			Norm.	Wilcoxon
	n	Median	IQR	Skewness	Statistic	d.f.	p	z	p
Physical Functioning	54	-0.29	2.03	-1.123	0.202	54	<0.001**	0	0.007**
Role Limitations - Physical	54	-0.45	1.76	-1.045	0.348	54	<0.001**	0	0.18
Global Health	54	0.95	1.29	-1.024	0.251	54	<0.001**	0	0.002**
Bodily Pain	54	-0.42	2.05	-0.457	0.182	54	<0.001**	0	0.12
Role Limits. - Emot./Behav.	54	0.49	0.71	-1.789	0.355	54	<0.001**	0	0.42
Parent Time	54	0.25	1.10	-1.808	0.280	54	<0.001**	0	0.55
Parent Emotion	54	0.13	0.96	-0.694	0.112	54	0.090	0	0.23
Self-Esteem	54	-0.71	2.34	0.079	0.133	54	0.018*	0	<0.001**
Mental	54	0.75	1.10	-1.949	0.212	54	<0.001**	0	<0.001**
Behaviour	54	0.74	2.33	-1.199	0.255	54	<0.001**	0	<0.001**

					Kolmogorov-Smirnov			Norm	1-sample t-test	
	n	Mean	Sd	Skewness	Statistic	d.f.	p	T	t	p
PhS	54	45.76	11.86	-1.269	0.101	54	0.200	50	-2.63	0.011*
PsS	54	51.26	8.05	-0.52	0.082	54	0.200	50	1.12	0.269

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.7 Description and one sample tests of CHQ-PF50 data

Description and one sample tests of CHQ-PF50 data (n=54)

			1-sample t-test		
	Mean	Sd	t	d.f.	p
Physical Functioning	-0.77	1.55	-3.67	53	0.001**
Role Limitations - Physical	-0.43	1.17	-2.70	53	0.01**
Global Health	0.40	1.07	2.76	53	0.01**
Bodily Pain	-0.38	1.17	-2.40	53	0.02
Role Limits. - Emot./Behav.	-0.08	0.95	-0.60	53	0.55
Parent Time	-0.01	1.06	-0.05	53	0.96
Parent Emotion	0.09	0.80	0.78	53	0.44
Self-Esteem	-0.61	1.14	-3.94	53	<0.001**
Mental	0.48	1.06	3.30	53	0.002**
Behaviour	0.19	1.50	0.93	53	0.36
Composite					
PhS	45.76	11.86	-2.63	53	0.011*
PsS	51.26	8.05	1.12	53	0.269

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.8 Median CHQ-PF50 z-scores

Table N: Description, tests of normality, and one sample tests of CHQ-PF50 data

					Kolmogorov-Smirnov			Norm.	Wilcoxon
	n	Median	IQR	Skewness	Statistic	d.f.	p	z	p
Physical Functioning	54	-0.29	2.03	-1.123	0.202	54	<0.001**	0	0.007**
Role Limitations - Physical	54	-0.45	1.76	-1.045	0.348	54	<0.001**	0	0.18
Global Health	54	0.95	1.29	-1.024	0.251	54	<0.001**	0	0.002**
Bodily Pain	54	-0.42	2.05	-0.457	0.182	54	<0.001**	0	0.12
Role Limits. - Emot./Behav.	54	0.49	0.71	-1.789	0.355	54	<0.001**	0	0.42
Parent Time	54	0.25	1.10	-1.808	0.280	54	<0.001**	0	0.55
Parent Emotion	54	0.13	0.96	-0.694	0.112	54	0.090	0	0.23
Self-Esteem	54	-0.71	2.34	0.079	0.133	54	0.018*	0	<0.001**
Mental	54	0.75	1.10	-1.949	0.212	54	<0.001**	0	<0.001**
Behaviour	54	0.74	2.33	-1.199	0.255	54	<0.001**	0	<0.001**

					Kolmogorov-Smirnov			Norm	1-sample t-test	
	n	Mean	Sd	Skewness	Statistic	d.f.	p	T	t	p
PhS	54	45.76	11.86	-1.269	0.101	54	0.200	50	-2.63	0.011*
PsS	54	51.26	8.05	-0.52	0.082	54	0.200	50	1.12	0.269

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.9 CHQ-PF50 and gender

CHQ-PF50 and Gender

	Median		Mann-Whitney				
	Female	Male	U	p			
Physical Functioning	-0.29	-0.46	310.0	0.793			
Role Limitations - Physical	0.45	0.45	322.0	0.967			
Global Health	0.95	0.95	286.0	0.458			
Bodily Pain	-0.42	-0.66	284.5	0.459			
Role Limits. - Emot./Behav.	0.49	-0.49	305.5	0.695			
Parent Time	0.25	0.25	299.0	0.630			
Parent Emotion	0.13	0.13	298.5	0.639			
Self-Esteem	-0.71	-1.64	346.5	0.152			
Mental	0.93	0.38	258.0	0.219			
Behaviour	0.74	0.01	230.5	0.075			
	Female		Male		t-test		
	Mean	Sd	Mean	Sd	t	df	p
PhS	45.65	13.29	45.97	8.66	-0.91	52	0.93
PsS	52.13	7.73	49.41	8.60	1.18	52	0.25

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.10 Statistical analysis of CHQ-PF50 scores by 3 LSL groups

Statistical analysis of CHQ-PF50 scores by 3 LSL groups									
	Kruskal-Wallis		Mann-Whitney		Mann-Whitney		Mann-Whitney	
	df=2, n=54		Caudal-Dorsal		Caudal-Transit.		Dorsal-Transit.	
	χ^2	*p*	U	*p*	U	*p*	U	*p*
Physical Functioning	3.996	0.136						
Role Limitations - Physical	0.463	0.793						
Global Health	5.875	0.053	138.5	0.883	121.5	0.04	91.5	0.038
Bodily Pain	11.770	0.003	136.5	0.827	87.0	0.003	65.5	0.04
Role Limits. - Emot./Behav.	1.261	0.532						
Parent Time	7.475	0.024	111.0	0.233	96.0	0.005	113.5	0.211
Parent Emotion	2.655	0.265						
Self-Esteem	2.208	0.332						
Mental	3.977	0.137						
Behaviour	8.070	.018	113.0	0.319	125.5	0.059	72.0	0.007

				Tukey HSD		Tukey HSD		Tukey HSD	
	1-way ANOVA			Caudal-Dorsal		Caudal-Transit.		Dorsal-Transit.	
	F	df	*p*	Mean diff.	*p*	Mean diff.	*p*	Mean diff.	*p*
PhS*	3.384	(2,51)	0.042	-0.891	0.972	7.904	0.086	8.795	0.07
PsS**	2.241	(2,51)	0.117						

Table 5.11 CHQ-PF50 and PAQ ratings

CHQ-PF50 and PAQ ratings	PAQ-C (n=11)		PAQ-A (n=23)		PAQ-C/A (n=34)	
	r_s	p	r_s	p	r_s	p
Physical Functioning	-0.18	0.59	0.53*	0.01	0.35*	0.04
Role Limitations - Physical	-0.05	0.89	0.20	0.36	0.10	0.56
Global Health	-0.49	0.13	0.19	0.39	-0.02	0.93
Bodily Pain	-0.07	0.85	0.28	0.20	0.20	0.26
Role Limits. - Emot./Behav.	-0.19	0.58	0.29	0.17	0.15	0.38
Parent Time	-0.03	0.92	0.32	0.14	0.16	0.36
Parent Emotion	-0.30	0.38	0.17	0.45	-0.01	0.97
Self-Esteem	0.19	0.57	0.39	0.06	0.30	0.08
Mental	0.00	0.99	0.40	0.06	0.27	0.12
Behaviour	0.02	0.96	0.09	0.68	0.07	0.68
PhS	-0.11	0.74	.445*	0.03	0.23	0.19
PsS	0.05	0.88	0.39	0.06	0.31	0.07

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.12 CHQ-PF50 and presence / absence of pain

CHQ-PF50 and presence/absence of pain

	No pain			Pain			Mann-Whitney	
	n	Median	IQR	n	Median	IQR	U	*p*
Physical Functioning	26	0.22	2.03	28	-0.80	2.46	271.0	0.10
Role Limitations - Physical	26	0.45	1.76	28	0.45	1.76	349.5	0.78
Global Health	26	0.95	1.49	28	0.95	1.29	340.5	0.67
Bodily Pain	26	0.06	1.45	28	-0.90	0.96	164.0**	<0.001
Role Limits. - Emot./Behav.	26	0.49	0.14	28	0.21	1.14	275.5	0.08
Parent Time	26	0.25	0.68	28	0.25	1.64	338.0	0.64
Parent Emotion	26	0.44	1.05	28	-0.07	0.90	241.5*	0.03
Self-Esteem	26	-0.71	2.34	28	-0.71	2.28	345.5	0.75
Mental	26	0.93	1.19	28	0.20	0.73	242.0	0.03
Behaviour	26	0.74	2.33	28	0.74	1.46	295.5	0.22

	No pain		Pain		t-test		
	Mean	Sd	Mean	Sd	t	df	*p*
PhS	48.66	10.58	43.07	12.53	1.77	52	0.08
PsS	52.55	9.22	49.99	6.72	1.17	52	0.25

* Significant at the 0.05 level

** Significant at the 0.01 level

Appendix table 5.13 CHQ-PF50 scores by 3 LSL groups

Statistical analysis of CHQ-PF50 scores by 3 LSL groups

	Caudal			Dorsal			Transitional			Kruskal-Wallis df=2, n=54		Mann-Whitney Caudal-Dorsal		Mann-Whitney Caudal-Transit.		Mann-Whitney Dorsal-Transit.	
	n	Median	IQR	n	Median	IQR	n	Median	IQR	χ^2	*p*	U	*p*	U	*p*	U	*p*
Physical Functioning	19	0.22	2.71	15	0.22	1.36	20	-0.80	3.56	3.996	0.136						
Role Limitations - Physical	19	0.45	1.76	15	0.45	1.76	20	0.01	1.76	0.463	0.793						
Global Health	19	0.95	2.07	15	0.95	2.07	20	-0.35	1.29	5.875	0.053	138.5	0.883	121.5*	0.04	91.5*	0.038
Bodily Pain	19	0.06	1.93	15	0.06	1.93	20	-0.90	1.33	11.770	0.003**	136.5	0.827	87.0**	0.003	65.5*	0.04
Role Limits. - Emot./ Behav.	19	0.49	0.57	15	0.49	1.71	20	0.49	1.57	1.261	0.532						
Parent Time	19	0.79	0.55	15	0.25	1.10	20	0.25	1.10	7.475	0.024*	111.0	0.233	96.0**	0.005	113.5	0.211
Parent Emotion	19	0.44	1.01	15	0.28	0.97	20	-0.07	1.27	2.655	0.265						
Self-Esteem	19	-0.47	1.40	15	-0.71	2.34	20	-1.17	2.28	2.208	0.332						
Mental	19	0.93	1.10	15	0.93	1.46	20	0.38	0.73	3.977	0.137						
Behaviour	19	0.74	2.33	15	1.62	0.87	20	-0.72	1.46	8.070	.018*	113.0	0.319	125.5	0.059	72.0**	0.007

	Caudal		Dorsal		Transitional		1-way ANOVA			Tukey HSD Caudal-Dorsal		Tukey HSD Caudal-Transit.		Tukey HSD Dorsal-Transit.	
	Mean	s.d.	Mean	s.d.	Mean	s.d.	F	df	*p*	Mean diff.	*p*	Mean diff.	*p*	Mean diff.	*p*
PhS	48.44	10.12	49.33	9.53	40.54	13.52	3.384	(2,51)	0.042	-0.891	0.972	7.904	0.086	8.795	0.07
PsS	52.63	7.68	53.35	6.65	48.29	8.82	2.241	(2,51)	0.117						

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.14 Statistical analysis of CHQ-PF50 scores by 3 LSL groups

Statistical analysis of CHQ-PF50 scores by 3 LSL groups

	Caudal			Dorsal			Transitional			Kruskal-Wallis df=2, n=54		Mann-Whitney Caudal-Dorsal		Mann-Whitney Caudal-Transit.		Mann-Whitney Dorsal-Transit.	
	n	Median	IQR	n	Median	IQR	n	Median	IQR	χ^2	*p*	U	*p*	U	*p*	U	*p*
Physical Functioning	19	0.22	2.71	15	0.22	1.36	20	-0.80	3.56	3.996	0.136						
Role Limitations - Physical	19	0.45	1.76	15	0.45	1.76	20	0.01	1.76	0.463	0.793						
Global Health	19	0.95	2.07	15	0.95	2.07	20	-0.35	1.29	5.875	0.053	138.5	0.883	121.5*	0.04	91.5*	0.038
Bodily Pain	19	0.06	1.93	15	0.06	1.93	20	-0.90	1.33	11.770	0.003**	136.5	0.827	87.0**	0.003	65.5*	0.04
Role Limits. - Emot./ Behav.	19	0.49	0.57	15	0.49	1.71	20	0.49	1.57	1.261	0.532						
Parent Time	19	0.79	0.55	15	0.25	1.10	20	0.25	1.10	7.475	0.024*	111.0	0.233	96.0**	0.005	113.5	0.211
Parent Emotion	19	0.44	1.01	15	0.28	0.97	20	-0.07	1.27	2.655	0.265						
Self-Esteem	19	-0.47	1.40	15	-0.71	2.34	20	-1.17	2.28	2.208	0.332						
Mental	19	0.93	1.10	15	0.93	1.46	20	0.38	0.73	3.977	0.137						
Behaviour	19	0.74	2.33	15	1.62	0.87	20	-0.72	1.46	8.070	.018*	113.0	0.319	125.5	0.059	72.0**	0.007

	Caudal		Dorsal		Transitional		1-way ANOVA			Tukey HSD Caudal-Dorsal		Tukey HSD Caudal-Transit.		Tukey HSD Dorsal-Transit.	
	Mean	s.d.	Mean	s.d.	Mean	s.d.	F	df	*p*	Mean diff.	*p*	Mean diff.	*p*	Mean diff.	*p*
PhS	48.44	10.12	49.33	9.53	40.54	13.52	3.384	(2,51)	0.042	-0.891	0.972	7.904	0.086	8.795	0.07
PsS	52.63	7.68	53.35	6.65	48.29	8.82	2.241	(2,51)	0.117						

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.15 Description and tests of normality of CHQ-CF87

Description and tests of normality of CHQ-CF87

					Kolmogorov-Smirnov		
	n	Median	IQR	Skewness	Statistic	d.f.	*p*
Global Health	25	85.00	40.00	-1.191	0.332**	25	<0.001
Physical Functioning	26	85.19	37.96	-1.05	0.211**	26	0.004
Role Limits. - Emotional	26	100.00	13.89	-1.62	0.310**	26	<0.001
Role Limits. - Behavioural	26	100.00	0.00	-4.72	0.486**	26	<0.001
Role Limits. - Physical	26	100.00	33.33	-1.98	0.396**	26	<0.001
Bodily Pain/Discomfort	26	70.00	30.00	-1.267	0.190*	26	0.016
Behaviour	26	87.79	18.24	-1.05	0.203**	26	0.007
Global Behaviour Item	26	85.00	40.00	-1.09	0.275**	26	<0.001
Mental Health	26	87.50	23.28	-1.113	0.262**	26	<0.001
Self-Esteem	27	76.79	35.71	-0.469	0.188*	27	0.016
General Health Perceptions	26	74.79	29.17	-0.77	0.147	26	0.155
Change in Health	26	4	2	-0.56	0.187*	26	0.020
Family Activities	26	83.33	38.54	-0.51	0.164	26	0.071
Family Cohesion	25	85.00	40.00	-0.91	0.249**	25	<0.001

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.16 Comparison of LSL and healthy normative CHQ-CF87 data (Raat et al, 2002)

	Estimate from mean			Estimate from % maximum			
	LSL	Normative		LSL	Normative		
	Median	Est. median	*p*	% max	% max	Est. median	*p*
Physical Functioning	85.19	96.8	0.001	23.1	60	100	<0.001
Role Limits. - Emotional	100.00	92.3	0.61	57.7	73	100	0.003
Role Limits. - Behavioural	100.00	91.4	<0.001	88.5	63	100	0.10
Role Limits. - Physical	100.00	96.5	0.91	69.2	86	100	0.01
Bodily Pain/Discomfort	70.00	78.2	0.004	3.8	30	-	
Behaviour	87.79	83.6	0.22	3.8	2	-	
Mental Health	87.50	78.2	0.26	0	1	-	
Self-Esteem	76.79	75.4	0.40	22.2	2	-	
General Health Perceptions	74.79	74.6	0.42	3.8	3	-	
Family Cohesion	85.00	75.7	0.07	44.0	30	-	

5.17 A discussion of statistical reasons for using the means / media in analysing the CHQ-CF87.

On the Role Limits – Behavioural scale, LSL patients appeared to have significantly higher scores when using the “Estimate from mean” method. However, this was not supported by comparison with the “Estimate from % maximum”. Finally, LSL patients reported significantly worse Bodily Pain/Discomfort than the norms, using the “Estimate from mean” method. The “Estimate from % maximum” method could not be applied, but comparison of percentages was consistent with this (3.8% compared to 30%). The use of multiple comparison corrections was considered and rejected, due to the exploratory nature of the current study. In a review of 6,415 abstracts for publication in ophthalmology research, Stacey et al {Stacey, 2012 #3018} suggest that multiple comparison corrections are not required in exploratory studies.

5.18 CHQ-CF87 and Gender

CHQ-CF87 and Gender

	n		Median		Mann-Whitney	
			Female	Male	U	*p*
Global Health	17	8	85.00	85.00	60.0	0.621
Physical Functioning	18	8	85.19	83.33	67.5	0.801
Role Limits. - Emotional	18	8	100.00	88.89	50.5	0.182
Role Limits. - Behavioural	18	8	100.00	100.00	70.0	0.841
Role Limits. - Physical	18	8	100.00	94.44	54.0	0.220
Bodily Pain/Discomfort	18	8	60.00	80.00	37.0*	0.045
Behaviour	18	8	89.26	87.35	65.0	0.697
Global Behaviour Item	18	8	85.00	85.00	56.5	0.362
Mental Health	18	8	87.50	87.97	70.5	0.933
Self-Esteem	18	8	89.29	67.86	66.5	0.453
General Health Perceptions	18	8	75.83	68.54	51.0	0.243
Change in Health	18	8	4.00	3.50	62.0	0.561
Family Activities	18	8	85.42	79.17	62.5	0.593
Family Cohesion	17	8	100.00	85.00	53.5	0.368

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.19 Statistical analysis of CHQ-CF87 scores by 3 LSL groups

Statistical analysis of CHQ-CF87 scores by 3 LSL groups								
	Kruskal-Wallis		Mann-Whitney		Mann-Whitney		Mann-Whitney	
	df=2		Caudal-Dorsal		Caudal-Transit.		Dorsal-Transit.	
	χ^2	*p*	U	*p*	U	*p*	U	*p*
Global Health	2.98	0.23						
Physical Functioning	3.99	0.14						
Role Limits. - Emotional	0.82	0.66						
Role Limits. - Behavioural	2.71	0.26						
Role Limits. - Physical	5.36	0.07						
Bodily Pain/Discomfort	0.51	0.78						
Behaviour	11.91**	<0.001	2.0	0.001	37.0	0.789	8.5	0.013
Global Behaviour Item	1.52	0.47						
Mental Health	6.04*	0.05	14.5	0.023	35.5	0.689	13.5	0.05
Self-Esteem	8.12*	0.02	10.5	0.008	34.5	0.390	13.0	0.025
General Health Perceptions	1.21	0.55						
Change in Health	0.90	0.64						
Family Activities	6.10*	0.05	32.5	0.495	16.5	0.035	12.5	0.038
Family Cohesion	3.99	0.14						

Table 5.20. Correlations between PedsQL and CHQ-CF87 ratings

Correlations between PedsQL-Self and CHQ-CF87 ratings (related constructs shaded)

		PedsQL-Self											
		Total		Physical		Psychosocial		Emotional		Social		School	
CHQ-CF87	*n*	r_s	*p*	r_s	*p*	r_s	*p*	r_s	*p*	r_s	*p*	r_s	*p*
Global Health	25	0.310	0.132	0.396*	0.050	0.266	0.199	0.334	0.103	0.146	0.486	0.168	0.422
Physical Functioning	26	0.727**	<0.001	0.858**	<0.001	0.618**	0.001	0.446*	0.022	0.413*	0.036	0.479*	0.013
Role Limits. - Emotional	26	0.300	0.136	0.271	0.181	0.338	0.091	0.412*	0.037	0.353	0.077	0.028	0.892
Role Limits. - Behavioural	26	-0.072	0.726	0.097	0.638	-0.179	0.383	-0.148	0.471	-0.155	0.451	-0.222	0.276
Role Limits. - Physical	26	0.439*	0.025	0.475*	0.014	0.419*	0.033	0.254	0.211	0.330	0.100	0.401*	0.042
Bodily Pain/Discomfort	26	0.333	0.096	0.445*	0.023	0.225	0.268	0.102	0.620	0.142	0.488	0.186	0.363
Behaviour	26	0.448*	0.022	0.444*	0.023	0.524**	0.006	0.344	0.085	0.478*	0.014	0.310	0.123
Global Behaviour Item	26	0.390*	0.049	0.254	0.211	0.457*	0.019	0.431*	0.028	0.254	0.210	0.462*	0.017
Mental Health	26	0.328	0.102	0.325	0.106	0.378	0.057	0.418*	0.034	0.417*	0.034	0.040	0.847
Self-Esteem	27	0.440*	0.022	0.405*	0.036	0.425*	0.027	0.325	0.098	0.511**	0.006	0.120	0.552
General Health Perceptions	26	0.535**	0.005	0.445*	0.023	0.471*	0.015	0.508**	0.008	0.436*	0.026	0.334	0.096
Change in Health	26	0.111	0.588	0.269	0.183	0.032	0.877	-0.058	0.777	0.076	0.713	0.093	0.651
Family Activities	26	0.534**	0.005	0.557**	0.003	0.470*	0.015	0.331	0.098	0.543**	0.004	0.227	0.265
Family Cohesion	25	0.614**	0.001	0.396*	0.050	0.635**	0.001	0.642**	0.001	0.523**	0.007	0.333	0.104

Correlations between PedsQL-Parent and CHQ-PF50 ratings (related constructs shaded)

		PedsQL-Proxy											
		Total		Physical		Psychosocial		Emotional		Social		School	
CHQ-PF50	*n*	r_s	*p*	r_s	*p*	r_s	*p*	r_s	*p*	r_s	*p*	r_s	*p*
Physical Functioning	54	0.595**	<0.001	0.687**	<0.001	0.487**	<0.001	0.374**	0.005	0.467**	<0.001	0.386**	0.004
Role Limitations - Physical	54	0.577**	<0.001	0.419**	0.002	0.573**	<0.001	0.494**	<0.001	0.445**	0.001	0.434**	0.001
Global Health	54	0.226	0.100	0.255	0.063	0.181	0.189	0.263	0.055	0.152	0.272	0.019	0.889
Bodily Pain	54	0.570**	<0.001	0.612**	<0.001	0.470**	<0.001	0.482**	<0.001	0.372**	0.006	0.273*	0.046
Role Limits. - Emot./Behav.	54	0.516**	<0.001	0.410**	0.002	0.492**	<0.001	0.538**	<0.001	0.250	0.068	0.420**	0.002
Parent Time	54	0.422**	0.001	0.299*	0.028	0.448**	0.001	0.402**	0.003	0.373**	0.005	0.465**	<0.001
Parent Emotion	54	0.265	0.053	0.145	0.295	0.293*	0.031	0.398**	0.003	0.169	0.221	0.155	0.262
Self-Esteem	54	0.219	0.112	0.231	0.092	0.173	0.212	0.213	0.122	0.155	0.264	0.053	0.706
Mental	54	0.445**	0.001	0.315*	0.020	0.469**	<0.001	0.490**	<0.001	0.369**	0.006	0.285*	0.037
Behaviour	54	0.123	0.376	0.017	0.903	0.165	0.233	0.283*	0.038	0.097	0.487	-0.061	0.659
PhS	54	0.721**	<0.001	0.719**	<0.001	0.623**	<0.001	0.536**	<0.001	0.541**	<0.001	0.468**	<0.001
PsS	54	0.273*	0.046	0.157	0.257	0.296*	0.030	0.466**	<0.001	0.144	0.300	0.120	0.387

Table 5 .21 Correlations between PedsQL parent and CHQ-PF50 ratings

Table 5.22 Description, tests of normality and one sample tests of PH2 data

Description, tests of normality and one sample tests of PH2 data (n=54)

				Kolmogorov-Smirnov			Wilcoxon (t test t-26)	
	Median	IQR	Skewness	Statistic	*df*	*p*	*p*	
Behavioural Adjustment	31	6	2.533	0.263	42	<0.001**	<0.001**	
Intellectual & Social Status	44	4	-0.549	0.260	42	<0.001**	<0.001**	
Physical Appearance & Attributes	45	10	0.174	0.206	42	<0.001**	<0.003**	
Freedom from Anxiety	35	13	0.466	0.144	42	0.028*	<0.001**	
Popularity	39	5.75	0.442	0.148	42	0.022*	<0.001**	
Happiness & Satisfaction	40	4	0.644	0.214	42	<0.001**	<0.001**	
							1-sample t-test	
	Mean	Sd	Skewness	Statistic	d.f.	*p*	*t*	*p*
Total	35.86	3.53	2.533	0.127	42	0.088	-26.01	<0.001**

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.23 Comparison of PH2 data to normative data using parametric methods

Scores were compared to a normative value of T = 50

:

			1-sample t-test		
	Mean	Sd	*t*	*d.f.*	*p*
Behavioural Adjustment	32.48	4.81	-23.59	41	<0.001**
Intellectual & Social Status	43.79	3.87	-10.40	41	<0.001**
Physical Appearance & Attributes	46.76	6.27	-3.35	41	0.002**
Freedom from Anxiety	35.74	7.20	-12.84	41	<0.001**
Popularity	37.83	4.13	-19.11	41	<0.001**
Happiness & Satisfaction	42.14	4.31	-11.80	41	<0.001**
Composite	Mean	Sd			
Total	35.86	3.53	-26.01	41	<0.001**

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.24 PH2 and Gender

PH2 and Gender (female n=28, male n=14)

	Median		Mann-Whitney	
	Female	Male	U	*p*
Behavioural Adjustment	31	31	177	0.60
Intellectual & Social Status	44	44	153	0.28
Physical Appearance & Attributes	45	45	180.5	0.67
Freedom from Anxiety	35	37	142	0.15
Popularity	36	39	140.5	0.13
Happiness & Satisfaction	43	40	160	0.32

	Female		Male		t-test		
	Mean	Sd	Mean	Sd	*t*	df	*p*
Total	35.54	3.42	36.50	3.78	0.83	40	0.41

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 5.25 Statistical analysis of PH2 scores by 3 LSL groups

Statistical analysis of PH2 scores by 3 LSL groups										
	Kruskal-Wallis		Mann-Whitney		Mann-Whitney		Mann-Whitney	
	df=2, n=42		Caudal-Dorsal		Caudal-Transit.		Dorsal-Transit.	
	χ^2	*p*	U	*p*	U	*p*	U	*p*
Behavioural Adjustment	0.043	0.98	-	-	-	-	-	-
Intellectual & Social Status	1.838	0.40	-	-	-	-	-	-
Physical Appearance & Attributes	0.17	0.92	-	-	-	-	-	-
Freedom from Anxiety	2.06	0.36	-	-	-	-	-	-
Popularity	4.54	0.10	-	-	-	-	-	-
Happiness & Satisfaction	0.03	0.98	-	-	-	-	-	-

				Tukey HSD		Tukey HSD		Tukey HSD	
	1-way ANOVA			Caudal-Dorsal		Caudal-Transit.		Dorsal-Transit.	
	F	df	*p*	Mean diff.	*p*	Mean diff.	*p*	Mean diff.	*p*
Total	2.07	2	0.14	-	-	-	-	-	-

Table 5.26 PH2 and NEM ratings

PH2 and NEM ratings (n=42)										
	NEM ratings									
	Motor		Sensory		Urology		Bowels		NEM_Total	
	r_s	p	r_s	p	r_s	p	r_s	p	r_s	p
Behavioural Adjustment	0.003	0.99	-0.04	0.79	-0.16	0.30	0.06	0.68	-0.10	0.54
Intellectual & Social Status	0.02	0.91	-0.05	0.77	0.09	0.56	-0.13	0.40	0.02	0.89
Physical Appearance & Attributes	0.10	0.52	0.11	0.50	0.09	0.57	-0.15	0.33	0.08	0.62
Freedom from Anxiety	-0.11	0.48	-0.21	0.19	-0.25	0.11	0.23	0.14	-0.16	0.30
Popularity	-0.03	0.83	-0.16	0.31	-0.06	0.69	0.27	0.08	0.03	0.85
Happiness & Satisfaction	-0.11	0.50	0.14	0.38	0.05	0.74	0.04	0.81	<0.001	1.00
Total	-0.06	0.68	-0.26	0.09	-0.39	0.01	0.16	0.30	-0.26	0.10

Table 5.27 PH2 and PAQ ratings (n=33)

	PAQ-C (n=10)		PAQ-A (n=23)		PAQ-C/A (n=33)	
	r_s	p	r_s	p	r_s	p
Behavioural Adjustment	0.09	0.80	-0.24	0.27	-0.14	0.44
Intellectual & Social Status	-0.06	0.87	-0.20	0.36	-0.15	0.41
Physical Appearance & Attributes	-0.27	0.44	0.11	0.61	0.04	0.81
Freedom from Anxiety	-0.03	0.94	-0.17	0.44	-0.11	0.54
Popularity	0.19	0.60	-0.26	0.23	-0.12	0.50
Happiness & Satisfaction	0.38	0.28	-0.16	0.47	-0.01	0.96
Total	<0.001	1.00	-0.38	0.07	-0.27	0.13

Appendix for chapter 7

Table 7.1 HADS and tests of normality

Description, and tests of normality of HADS data (n=54)

				Kolmogorov-Smirnov		
	Median	IQR	Skewness	Statistic	*df*	*P*
HADS-A	8	6	0.787	0.118	54	0.058
HADS-D	3	6	1.106	0.173	54	<0.001

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 7.2 HADS and Gender

Parental HADS and child gender (female n=36, male n=18)

	Median		Mann-Whitney	
	Female	Male	U	*p*
HADS-A	8	8	305.0	0.73
HADS-D	3	3	306.0	0.74

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 7.3 HADS and the presence of a syrinx

HADS and the presence of a syrinx

	Median		Mann-Whitney	
	No syrinx *n*=38	Syrinx *n*=16	U	*p*
HADS-A	7.5	9.0	240.0	0.22
HADS-D	3.0	3.0	297.5	0.90

Table 7.4 HADS and LSL type

: Parental HADS and LSL type

	Caudal			Dorsal			Transitional			Kruskal-Wallis (df=2)	
	n	Median	IQR	n	Median	IQR	n	Median	IQR	χ^2	*p*
HADS-A	19	7.00	6.00	15	8.00	4.00	20	9.00	6.75	2.18	0.34
HADS-D	19	2.00	7.00	15	3.00	4.00	20	4.50	8.25	0.81	0.67

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 7.5 HADS and the presence / absence of pain

HADS and presence/absence of pain

	No pain			Pain			Mann-Whitney	
	n	Median	IQR	n	Median	IQR	U	*p*
HADS-A	26	7.5	7.00	28	8.5	5.00	318.0	0.42
HADS-D	26	2.5	5.00	28	3.5	7.75	298.0	0.25

Table 7.6 HADS and physical activity (PAQ-C and PAQ-A ratings)

	PAQ-C (n=11)		PAQ-A (n=23)		PAQ-C/A (n=34)	
	r_s	p	r_s	p	r_s	p
HADS-A	-0.25	0.46	0.15	0.48	0.04	0.82
HADS-D	-0.25	0.46	-0.09	0.68	-0.13	0.48

Table 7.7 PIP and Tests of normality

Description, and tests of normality of PIP data (n=54)

					Kolmogorov-Smirnov		
		Median	IQR	Skewness	Statistic	*df*	*p*
Communication	Frequency	17.0	11.25	0.443	0.134*	54	0.02
	Difficulty	15.5	12.0	0.452	0.131*	54	0.02
Emotional Distress	Frequency	29.5	20.5	0.442	0.117	54	0.06
	Difficulty	31.0	21.75	0.496	0.147**	54	0.005
Medical Care	Frequency	15.0	13.5	0.450	0.142**	54	0.008
	Difficulty	14.5	13.25	0.668	0.166**	54	<0.001
Role Function	Frequency	17.0	14.0	0.735	0.141**	54	0.01
	Difficulty	17.5	13.50	0.611	0.159**	54	0.002
Total	Frequency	76.5	51.75	0.460	0.126*	54	0.03
	Difficulty	76.5	56.25	0.459	0.133*	54	0.02

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 7.8 PIP and gender

Parental PIP and child gender (female n=36, male n=18)

		Median		Mann-Whitney	
		Female	Male	U	*p*
Communication	Frequency	18.5	15.0	294.0	0.58
	Difficulty	18.0	14.0	299.0	0.65
Emotional Distress	Frequency	29.5	28.5	322.5	0.98
	Difficulty	31.0	30.0	321.0	0.96
Medical Care	Frequency	15.5	12.5	300.0	0.66
	Difficulty	15.5	12.0	308.0	0.77
Role Function	Frequency	17.0	18.5	311.5	0.82
	Difficulty	18.0	16.5	321.5	0.96
Total	Frequency	80.0	72.5	317.0	0.90
	Difficulty	84.5	72.0	316.0	0.88

Table 7.9 PIP and syrinx

PIP and Syrinx

		Median		Mann-Whitney	
		No syrinx	Syrinx		
		n=38	*n*=16	U	*p*
Communication	Frequency	16.0	19.5	283.5	0.70
	Difficulty	15.0	18.5	266.5	0.48
Emotional Distress	Frequency	28.5	31.0	274.0	0.57
	Difficulty	29.0	37.0	264.0	0.45
Medical Care	Frequency	14.0	19.5	235.5	0.19
	Difficulty	12.0	17.0	253.5	0.34
Role Function	Frequency	17.0	18.5	274.5	0.57
	Difficulty	17.0	19.5	269.5	0.51
Total	Frequency	73.5	90.0	268.0	0.50
	Difficulty	72.0	96.0	262.5	0.43

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 7.10 PIP and pain

PIP and pain

		No pain			Pain			Mann-Whitney	
		n	Median	IQR	n	Median	IQR	U	*p*
Communication	Frequency	26	14.0	12.00	28	18.5	10.50	272.0	0.11
	Difficulty	26	14.5	12.50	28	17.0	11.00	301.0	0.27
Emotional Distress	Frequency	26	28.0	19.50	28	31.0	20.50	294.0	0.23
	Difficulty	26	27.0	27.75	28	32.5	20.25	306.5	0.32
Medical Care	Frequency	26	12.5	12.50	28	18.0	15.25	262.5	0.08
	Difficulty	26	10.0	13.50	28	15.0	11.75	266.0	0.09
Role Function	Frequency	26	15.5	12.75	28	18.0	15.25	315.0	0.39
	Difficulty	26	13.5	14.25	28	20.5	14.75	292.0	0.21
Total	Frequency	26	71.5	59.25	28	77.0	52.25	281.0	0.15
	Difficulty	26	70.0	62.00	28	82.5	47.75	287.5	0.19

* Significant at the 0.05 level

** Significant at the 0.01 level

Table 7.11 PIP and activity ratings

PIP and PAQ ratings

		PAQ-C (n=11)		PAQ-A (n=23)		PAQ-C/A (n=34)	
PIP		r_s	p	r_s	p	r_s	p
Communication	Frequency	-0.10	0.76	-0.06	0.78	-0.07	0.71
	Difficulty	-0.16	0.64	-0.17	0.45	-0.17	0.34
Emotional Distress	Frequency	-0.07	0.85	-0.34	0.11	-0.25	0.15
	Difficulty	-0.29	0.38	-0.40	0.06	-0.29	0.10
Medical Care	Frequency	-0.23	0.49	-0.12	0.58	-0.14	0.44
	Difficulty	-0.28	0.40	-0.16	0.47	-0.17	0.34
Role Function	Frequency	-0.31	0.36	-0.18	0.41	-0.20	0.25
	Difficulty	-0.40	0.23	-0.23	0.30	-0.27	0.13
Total	Frequency	-0.12	0.72	-0.17	0.44	-0.16	0.38
	Difficulty	-0.33	0.32	-0.26	0.23	-0.22	0.21